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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2022
OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from _____ to _____
Commission File Number: 001-37625

Voyager Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**46-3003182**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification No.)**
64 Sidney Street, Cambridge, Massachusetts	**02139**
(Address of Principal Executive Offices)	**(Zip Code)**

(857) 259-5340
(Registrant's Telephone Number, Including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	VYGR	Nasdaq Global Select Market

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. **Yes** ☐ **No** ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. **Yes** ☐ **No** ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. **Yes** ☒ **No** ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). **Yes** ☒ **No** ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). **Yes** ☐ **No** ☒

The aggregate market value of Common Stock held by non-affiliates of the registrant computed by reference to the price of the registrant's Common Stock as of June 30, 2022, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $113.6 million (based on the last reported sale price on the Nasdaq Global Select Market as of such date).

As of March 1, 2023, there were 43,293,369 shares of the registrant's common stock, par value $0.001 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement relating to its 2023 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. Such Proxy Statement is expected to be filed with the U.S. Securities and Exchange Commission not later than 120 days after the end of the fiscal year to which this report relates.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "contemplate," "anticipate," "goals," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, among other things, statements about:

- our plans to develop and commercialize our product candidates based on adeno-associated virus, or AAV, gene therapy and our proprietary antibodies;
- our ability to continue to develop our proprietary gene therapy platform technologies, including our TRACER™ discovery platform and our vectorized antibody platform, and our proprietary antibodies;
- our ability to identify and optimize product candidates and proprietary AAV capsids;
- our strategic collaborations with and funding from our collaboration partner Neurocrine Biosciences, Inc., or Neurocrine, from our option and license arrangement with Pfizer Inc., or Pfizer, and from our option and license arrangement with Novartis Pharma AG, or Novartis;
- our ongoing and planned preclinical development efforts, related timelines and studies;
- our ability to enter into future collaborations, strategic alliances, or option and license arrangements;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for our product candidates, including the ability to file investigational new drug, or IND, applications for our programs;
- our estimates regarding expenses, contingent liabilities, future revenues, existing cash resources and capital requirements;
- our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection for our proprietary assets;
- our estimates regarding the size of the potential markets for our product candidates and our ability to serve those markets;
- our need for additional funding and our plans and ability to raise additional capital, including through equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements;
- our competitive position and the success of competing products that are or become available for the indications that we are pursuing;
- the impact of government laws and regulations including in the United States, the European Union, and other important geographies such as Japan; and
- our ability to control costs and prioritize our product candidate pipeline successfully in connection with our strategic initiatives.

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements. You should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in "Part I, Item 1A - Risk Factors" that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, strategic collaborations, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to the Annual Report on Form 10-K with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

RISK FACTOR SUMMARY

Investment in our securities involves risk and uncertainties that you should be aware of when evaluating our business. The following is a summary of what we believe to be the principal risks facing our business, as more fully described under " Part I, Item 1A - Risk Factors" and elsewhere in this Annual Report on Form 10-K. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations.

- We have a history of incurring significant losses and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain consistent profitability. We may not be able to generate sufficient revenue from the commercialization of our product candidates and may never be consistently profitable.
- We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.
- Our AAV gene therapy and other biological therapy product candidates are based on a proprietary technology and, in several disease areas, unvalidated treatment approaches, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and subsequently obtaining regulatory approval for, our product candidates.
- Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.
- We are early in our development efforts. All of our active product candidates are currently in preclinical development. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.
- Our product candidates or the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

- We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.
- To date, all of our revenue has been derived from our ongoing collaborations with Neurocrine, from our ongoing option and license arrangements with Pfizer and Novartis, and from our prior collaborations with Sanofi Genzyme Corporation, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company. If any ongoing or future collaboration or option and license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.
- Our gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.
- Our future success depends on our ability to retain key members of our management and research and development teams, and to attract, retain and motivate qualified personnel.
- Our gene therapy and vectorized antibody approaches utilize vectors derived from viruses that are selectively engineered, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.
- If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

PART I

ITEM 1. BUSINESS

We are a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. We believe the potential of both disciplines has been constrained by delivery challenges; we are leveraging expertise in capsid discovery and neuropharmacology to address these constraints. Our gene therapy platforms enable us to engineer, optimize, manufacture and deliver AAV based gene therapies that we believe have the potential to safely provide durable efficacy. Our team of experts in the field of AAV gene therapy and neuroscience first identifies and selects diseases in which we believe an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. We then engineer and optimize an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

We are identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. Our team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood brain barrier, or BBB. The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates. We believe that capsids we discover through our TRACER discovery platform, which we refer to as TRACER capsids, have the potential to significantly enhance the efficacy and safety of our single dose gene therapies, which we expect to be delivered with systemic infusions, as compared with conventional capsids.

In addition to leveraging TRACER capsids in potential licensing arrangements, we are advancing our own proprietary pipeline of drug candidates for neurological diseases. Our wholly-owned prioritized pipeline programs include: superoxide dismutase 1, or SOD1, gene therapy for amyotrophic lateral sclerosis, or ALS, and an anti-tau antibody for Alzheimer's disease. We have identified a lead development candidate for our anti-tau antibody program and we expect to identify a lead development candidate for our SOD1 program during the first half of 2023. We expect to file INDs for both programs in 2024. In addition to these two wholly-owned programs, we are actively advancing two programs in collaboration with Neurocrine: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a FXN gene therapy program for Friedreich's ataxia. We also maintain a robust early research pipeline of wholly-owned and collaborative gene therapy programs for neurological diseases.

Mission and Strategy

Our mission is to is leverage our expertise in neuroscience and our pioneering discoveries in AAV capsids to advance life-changing gene therapies and other therapeutic modalities for neurological diseases. Our strategy to achieve this mission is to:

- ***Continuously advance in the development of our AAV gene therapy platform***. We plan to continuously invest in our gene therapy platform to maintain our strong position in the development of next-generation AAV gene therapies for neurological disorders and other serious diseases.

- ***Optimize and advance our gene therapy programs***. We have a pipeline comprised of a variety of preclinical programs that we intend to enable with proprietary, next-generation AAV capsids.

- ***Partner and collaborate to maximize the opportunities for our pipeline of gene therapy programs focused on severe neurological diseases and other serious diseases***. We believe that our experience in AAV gene therapy for severe neurological diseases, our pipeline of gene therapies, and our gene therapy platform provide us with the opportunity to collaborate to enhance our portfolio's long-term value.

- ***Partner with gene therapy developers to make available AAV capsids identified by our TRACER system***. We expect to make these capsids available through potential licensing agreements and other arrangements.

- ***Establish a leadership position in high quality AAV manufacturing***. We believe that manufacturing capacity and expertise are critical to successfully treating patients using gene therapy.

- ***Retain commercialization rights to select pipeline programs.*** We hold worldwide rights to our proprietary pipeline programs for various diseases and have retained certain commercialization rights for other programs.

- ***Expand our intellectual property portfolio***. We seek to have an industry-leading intellectual property portfolio across all facets of our business, including vector engineering and construct design, proprietary capsids, our production process, the compositions and methods of delivery of our product candidates.

AAV Gene Therapy

Gene therapy is an approach whereby gene expression is directly altered in patients to address the underlying cause or predominant manifestations of disease. We believe that the targeted nature of gene therapy may enable powerful treatment options and provide these patients with meaningful and durable benefits.

While AAV gene therapy can potentially be harnessed for multiple treatment methods, we are currently focused on gene replacement, gene knockdown and vectorized antibody approaches. Gene replacement is intended to restore the expression of a protein that is not expressed, expressed at abnormally low levels or functionally mutated with loss of function. Gene knockdown, or gene silencing, is intended to reduce the expression of a pathologically mutated RNA or protein that has detrimental effects. Vectorizing an antibody for delivery using AAV has the ability to increase exposure of large antibodies in brain parenchyma and interstitial fluid that otherwise show minimal penetration across the BBB when administered passively. Our gene therapy approach uses AAV vectors which we believe are ideal vectors for gene therapy for several reasons:

- ***Broad Applicability.*** AAV is able to transduce, or transfer a therapeutic gene, into numerous cell types including target cells in the central nervous system, or CNS, cardiac, and other tissues.

- ***Safety.*** We believe AAV is safe and is not known to cause any disease in humans.

- ***Does Not Readily Integrate.*** AAV does not readily integrate into the genome of the target cell, an attribute which we believe reduces the potential for oncogenesis, or the induction of cancer.

- ***Scalability.*** AAV is able to be manufactured at commercial quality and scale.

We believe that neurological diseases are well-suited for treatment with AAV gene therapy for the following reasons:

- ***Validated Targets.*** Many neurological, cardiac, and other diseases are caused by well-defined mutations in genes and these genes represent genetically validated drug targets for AAV gene therapy.

- ***Targeted Delivery.*** We believe our TRACER capsids may allow for significantly enhanced gene therapy delivery to specific types of cells and tissues at lower doses.

- ***Durable Expression.*** Long-term gene expression may be achievable in the CNS and other tissues following one-time dosing and transfer of the therapeutic gene with an AAV vector. Because repeated or continual dosing with direct injection of drugs into the CNS and other tissues is complex, a one-time AAV gene therapy has significant advantages.

The Voyager Gene Therapy Platform

We have built a gene therapy platform that we believe positions us to be the leading company at the intersection of AAV gene therapy and neurological diseases. Our team of experts in the field of AAV gene therapy first identifies and selects diseases that are well-suited for treatment using AAV gene therapy. We then engineer and optimize AAV vectors, identifying a capsid for delivery of a payload, comprising a therapeutic gene or transgene, and a promoter to drive expression of the transgene, to the targeted tissue or cells. Finally, we leverage established routes of administration and advances in dosing techniques to optimize delivery of our AAV vectors to target cells that are critical to the disease of interest. We believe that optimizing each of these parameters is a key factor for overall program success. We expect that our current and future pipeline programs will make use of technological advances generated with our gene therapy platform.

Disease Selection

Following an internal review process, we have prioritized pipeline programs for our development. This review evaluated the opportunity presented by each prioritized program based on the following criteria: high unmet medical need, target validation, efficient path to human proof of biology, robust preclinical pharmacology, and strong commercial potential.

Vector Engineering and Optimization

The key components of an AAV vector include: (a) the capsid; (b) the therapeutic gene, or transgene; and (c) payload control elements, including the promoter or other DNA sequences that modulate the expression of the transgene. We have advanced or intend to advance our multiple preclinical programs towards selection of lead clinical candidates using AAV vectors that we believe are best suited for each of our programs either through use of our existing capsids, through exercising a non-exclusive worldwide commercial license to capsid sequences covered by third parties, or by engineering or optimizing TRACER capsids. We have also built, or intend to build, capabilities to design, screen, and advance genetic sequences within our AAV vectors, including transgenes and payload control elements, to create optimized therapeutic candidates for each of our preclinical programs.

TRACER Capsid Discovery

Our scientists have developed TRACER, a proprietary AAV capsid discovery platform to facilitate the selection of TRACER capsids for particular therapeutic applications based on BBB-crossing and cell-specific transduction properties in multiple species, including non-human primates, or NHPs. We believe these TRACER capsids may allow for significantly enhanced gene delivery to specific types of cells in the brain at lower doses and, potentially, with fewer safety and tolerability issues than first-generation therapies. These TRACER capsids are now in advanced stages of characterization for deployment in our gene therapy development programs. We continue to perform screening campaigns with our TRACER discovery platform to identify additional proprietary AAV9- and AAV5-derived TRACER capsids and to refine previously-identified TRACER capsids to target or de-target multiple tissue and cell types.

We are actively engaged in discussions to make TRACER capsids available to third parties for use in their drug development programs through potential option and license and other arrangements. We believe there is significant opportunity for option and license transactions related to our TRACER capsids. To maximize the potential of our TRACER capsids for both our own programs and option and license transactions, we have retained to date, and expect to retain in the future, all rights associated with such TRACER capsids other than the rights specific to their use in combination with the optionee's or licensee's transgenes or collaborators' programs.

Collaboration Agreements

In January 2019, we entered into a collaboration with Neurocrine, or the 2019 Neurocrine Collaboration Agreement, for the research, development and commercialization of certain of our AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of four collaboration programs, which we refer to collectively as the 2019 Neurocrine Programs: the NBIb-1817 (VY-AADC) program, or the VY-

AADC Program for the treatment of Parkinson's disease, the program for the treatment of Friedreich's ataxia, or the FA Program, including the development of the VY-FXN01 product candidate, which together with the VY-AADC Program, we refer to as the Legacy Programs, and other undisclosed programs, or the 2019 Discovery Programs and, collectively with the Legacy Programs, the 2019 Neurocrine Programs. In August 2021, the collaboration was terminated with respect to the VY-AADC Program. Under the FA Program, we and Neurocrine are currently developing a gene therapy for the treatment of Friedreich's ataxia, a debilitating neurodegenerative disease resulting in poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing, and speech. Development of the two targets approved by the joint steering committee under the 2019 Discovery Program will continue into 2023.

On January 8, 2023, we entered into a collaboration and license agreement with Neurocrine, or the 2023 Neurocrine Collaboration Agreement, for the research, development, manufacture and commercialization of gene therapy products directed to the gene that encodes GBA1 for the treatment of Parkinson's disease and other diseases associated with GBA1, or the GBA1 Program, and three new programs focused on the research, development, manufacture and commercialization of gene therapies designed to address CNS diseases or conditions associated with rare genetic targets, or the 2023 Discovery Programs and, collectively with the GBA1 Program, the 2023 Neurocrine Programs. The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023. For more information, refer to Note 15 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

License Agreements

In October 2021, we entered into an option and license agreement with Pfizer, or the Pfizer Agreement, pursuant to which we granted Pfizer options to receive an exclusive license, or the Pfizer License Options, to certain TRACER capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes, or Pfizer Transgenes.

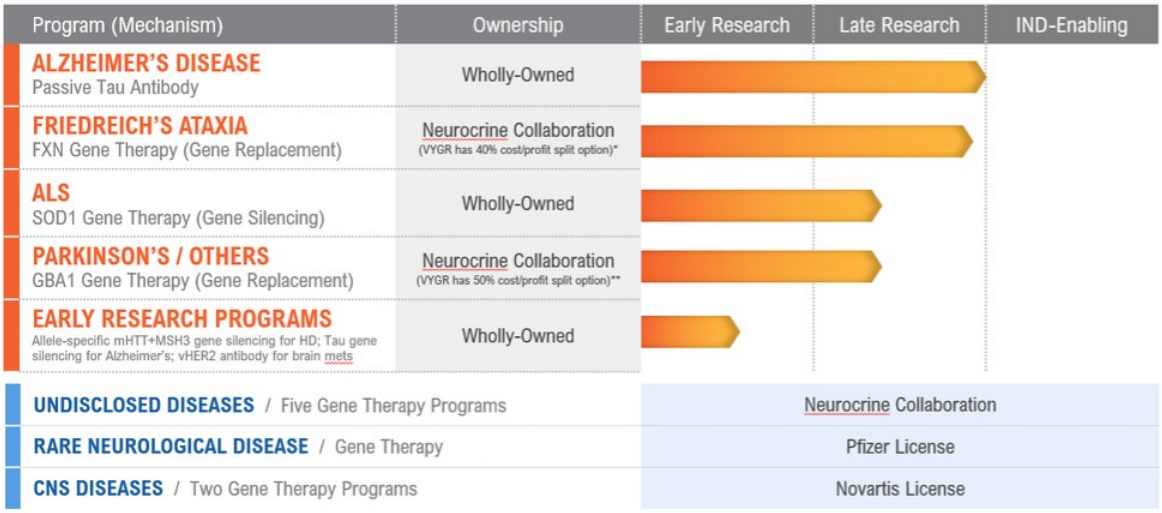
In March 2022, we entered into an option and license agreement, or the Novartis Agreement, with our collaborative partner Novartis. Pursuant to the Novartis Agreement, we have granted Novartis options, or the Novartis License Options, to license TRACER capsids, or the Novartis Licensed Capsids, for exclusive use with certain targets to develop and commercialize certain adeno-associated virus gene therapy candidates comprised of a Novartis Licensed Capsid and a payload directed to such target, or a Novartis Payload.

In November 2022, we and Touchlight IP Limited, or Touchlight, entered into a license agreement, or the Touchlight License Agreement, to authorize historical use by us of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER capsids created with the use of the Subject DNA Preparation Process.

Overview of Our Pipeline

We have leveraged our TRACER discovery platform and other gene therapy platforms, our expertise with proprietary antibodies, and our vectorized antibody platform to assemble a pipeline of proprietary AAV gene therapies and passive and vectorized payloads for the treatment of neurological and other diseases which we believe have high unmet medical need. Depending on the disease, we are seeking to develop AAV gene therapies that will use a gene replacement or gene silencing approach, and antibodies that will use a passive administration or vectorized delivery approach. Our goal is to address the underlying cause or the predominant manifestations of specific diseases by significantly increasing or decreasing expression of the relevant proteins in targeted tissues.

Our pipeline of our programs, all of which are in preclinical development, is summarized in the table below:



*After the Phase 1 readout, Voyager has the option to either: (1) co-develop and co-commercialize with Neurocrine Biosciences in the United States under a 60/40 cost- and profit-sharing arrangement (Neurocrine/Voyager), or (2) grant Neurocrine Biosciences full United States commercial rights in exchange for milestone payments and royalties based on United States sales.
** After the Phase 1 readout, Voyager has the option to either: (1) co-develop and co-commercialize with Neurocrine Biosciences in the United States under a 50/50 cost- and profit-sharing arrangement, or (2) grant Neurocrine Biosciences full United States commercial rights in exchange for milestone payments and royalties based on United States sales.

Anti-Tau Antibody Program for the Treatment of Alzheimer's Disease

Disease Overview

We are developing proprietary antibodies that selectively target and reduce the spread of pathological tau for the treatment of tauopathies, and our lead indication is Alzheimer's disease, or AD. The spread of tau pathology closely correlates with disease progression and cognitive decline in AD, which affects approximately 6 million people in the United States, and is a growing health care burden to society. Recently, anti-amyloid antibodies have been approved for treatment of AD, and there is substantial remaining unmet medical need.

Our Treatment Approach

We have maintained a long-standing focus on developing proprietary and complimentary approaches to disrupt the progression of tau pathology believed to be central to AD and other tauopathies. Reduction of toxic tau aggregates may slow disease progression and cognitive decline in these diseases. We are exploring passive administration of our anti-tau antibody. Our anti-tau antibodies have differentiated properties including improved targeting of specific regions of tau protein that could offer an improved profile compared to first-generation approaches. We believe that our antibody targeting the C-terminus is highly differentiated from other approaches. Further, we believe that following the clearance of an IND application, clinical assessments utilizing positron emission tomography (PET) imaging of human tau, together with measuring plasma and cerebrospinal fluid biomarkers, have the potential to enable an efficient and accelerated demonstration of human proof-of-biology.

Preclinical Studies

At the Alzheimer's Association International Conference in August 2022, we presented data for our proprietary anti-tau antibodies, targeting the mid-domain and C-terminus with high affinity and showing favorable biophysical characteristics and strong activity in preclinical studies in mouse models. In the P301S seeding-propagation tauopathy

mouse model, our C-terminal targeting anti-tau antibody blocked the seeding/propagation of filamentous tau and demonstrated substantial reduction of induced tau pathology.

Program Status

In January 2023, we selected a lead humanized anti-tau antibody candidate to advance against AD. The lead candidate, VY-TAU01, targets the C-terminal domain. VY-TAU01 was selected for its affinity, selectivity, and biophysical characteristics. Process development and manufacturing at a contracted manufacturer have been initiated, and we expect to initiate a good laboratory practices, or GLP, toxicology study later in 2023 to enable an IND filing in the first half of 2024.

Friedreich's Ataxia Program: VY-FXN01 (2019 Neurocrine Collaboration)

Disease Overview

Friedreich's ataxia is a debilitating neurodegenerative disease resulting in poor coordination of legs and arms, progressive loss of the ability to walk, generalized weakness, loss of sensation, scoliosis, diabetes and cardiomyopathy as well as impaired vision, hearing and speech. The typical age of onset is 10 to 12 years, and life expectancy is severely reduced with patients generally dying of neurological and cardiac complications between the ages of 35 and 45. According to the Friedreich's ataxia Research Alliance, there are approximately 6,400 patients living with the disease in the United States. While one treatment for Friedreich's ataxia has recently been approved by the FDA, there remains a significant unmet need.

Friedreich's ataxia patients have mutations of the FXN gene that reduce production of the frataxin protein, resulting in the degeneration of sensory pathways and a variety of debilitating symptoms. Friedreich's ataxia is an autosomal recessive disorder, meaning that a person must obtain a defective copy of the FXN gene from both parents in order to develop the condition. One healthy copy of the FXN gene, or 50% of normal frataxin protein levels, is sufficient to prevent the disease phenotype. We therefore believe that restoring FXN protein levels to at least 50% of normal levels by AAV gene therapy might lead to a successful therapy.

Our Treatment Approach

We are seeking to develop an AAV gene therapy approach that we believe will deliver a functional version of the FXN gene to the sensory pathways through intravenous injection. We think this approach has the potential to improve balance, ability to walk, sensory capability, coordination, strength and functional capacity of Friedreich's ataxia patients. Most Friedreich's ataxia patients produce low levels of the frataxin protein, which although insufficient to prevent the disease, exposes the patient's immune system to frataxin. This reduces the likelihood that the FXN protein expressed by AAV gene therapy will trigger a harmful immune response.

Preclinical Studies

We initially conducted preclinical studies in non-human primates and achieved high FXN expression levels within the target sensory ganglia, or clusters of neurons, along the spinal region following intrathecal injection. More recently, we conducted preclinical studies in non-human primates with IV injection and achieved target FXN expression levels within sensory ganglia and the heart. The levels of FXN expression observed in the brain using an AAV vector were, on average, greater than FXN levels present in control normal human brain tissue. FXN expression was also observed in the cerebellar dentate nucleus, another area of the CNS that is often affected in Friedreich's ataxia, and that is often considered difficult to target therapeutically.

Our Program Status

As part of the 2019 Neurocrine Collaboration, we are developing VY-FXN01 for the treatment of Friedreich's ataxia. VY-FXN01 is currently in preclinical development. We and Neurocrine are in the process of identifying a lead candidate that will comprise a capsid, promoter, and FXN transgene and are evaluating the potential use of TRACER

capsids in the program. We are completing AAV capsid biodistribution experiments to confirm capsid serotypes that effectively transduce disease target tissues in non-human primates following intravenous injection. Criteria for evaluating these capsids include safety, the overall level of transgene expression achieved, and the anatomic and cellular distribution of the transgene expression. Also, we have optimized the promoter for VY-FXN01 to achieve an acceptable therapeutic index for frataxin replacement. To evaluate the therapeutic potential of our vectors, we have conducted testing in a new genetic mouse model of Friedreich's ataxia. In this preclinical model of Friedreich's ataxia, our gene therapy candidates durably improved sensory function and rescued the disease phenotype based on multiple functional tests. In physiological and behavioral assays, our gene therapy candidates demonstrated dose-dependent and durable responses for more than 10 months after a single administration, preventing central and peripheral disease progression. We also have a significant effort focused on better understanding the clinical course of Friedreich's ataxia, identifying potential fluid biomarkers and selecting clinical endpoints for future clinical trials. As part of our portfolio reevaluation and strategic shift to invest in novel capsid development efforts, we and Neurocrine are evaluating the potential use of our TRACER capsids to allow for enhanced transduction across the disease target tissues. If we and Neurocrine successfully identify a development candidate and capsid for this program, we plan to complete IND enabling studies to evaluate its safety and efficacy.

SOD1 Gene Silencing Program for the Treatment of ALS

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER capsid to treat ALS caused by the SOD1 mutation via a gene silencing approach. SOD1 ALS is typically fatal within approximately three years of diagnosis and impacts approximately 800 patients in the United States, 1,000 patients in the European Union, and 500 patients in Japan. SOD1 mutations in ALS patients are thought to cause a toxic gain-of-function that leads to the degeneration of motor neurons along the entire length of the spinal cord, the brainstem, and the upper motor neurons in the cerebral cortex.

Our Treatment Approach

We believe that a therapeutic delivering a vectorized highly potent small interfering RNA, or siRNA, construct via intravenous administration of an AAV gene therapy with a vectorized siRNA may enable broad CNS knockdown of SOD1, potentially slowing the decline of functional ability in ALS patients with the SOD1 mutation. We believe that a Phase 1 clinical trial to demonstrate reduction in SOD1 in cerebrospinal fluid and neurofilament light chain in plasma will provide evidence of target engagement and the attenuation of motor neuron loss, respectively.

Preclinical Studies

At the ASGCT 2022 Meeting, we presented preclinical data demonstrating robust SOD1 knockdown in all levels of the spinal cord and significant improvements in motor performance, body weight, and survival in an SOD1-ALS mouse model following intravenous delivery of a vectorized siRNA using a mouse BBB-penetrant capsid.

Program Status

We have identified a potent and specific vectorized siRNA transgene that resulted in substantially extended lifespan and motor function when delivered using a BBB-penetrant capsid in an animal model. We are currently in the process of selecting a TRACER capsid with BBB-penetration activity in NHP studies for selection of a lead candidate vector.

GBA1 Gene Replacement Program for the Treatment of Parkinson's Disease (2023 Neurocrine Collaboration)

Disease Overview

We are developing a gene therapy leveraging a BBB-penetrant, CNS-tropic TRACER capsid to treat diseases linked to GBA1 mutations via a gene replacement approach. Our lead indication for this gene therapy is Parkinson's

disease with GBA1 mutations. Mutations in GBA1, the gene encoding the lysosomal glucocerebrosidase enzyme, or Gcase, are the most common genetic risk factor for synucleinopathies such as Parkinson's disease. Parkinson's disease is among the most common neurodegenerative diseases, impacting about one million patients in the United States and more than 10.0 million patients worldwide. Up to 10% of Parkinson's disease patients have a GBA1 mutation, and these mutations increase the risk of Parkinson's disease by approximately 20-fold. GBA1 mutations can decrease the activity of Gcase, leading to the accumulation of Gcase substrates which is linked to alpha-synuclein aggregates, that are thought to be toxic to neurons.

Our Treatment Approach

We believe that restoring Gcase activity may attenuate disease progression and potentially slow neurodegeneration. We anticipate delivering GBA1 via intravenous administration of an AAV gene therapy to enable widespread distribution to multiple affected brain regions and to avoid the need for more invasive approaches. We believe that the measurement of the Gcase substrates such as glucosylsphingosine as cerebrospinal fluid biomarkers may facilitate efficient clinical demonstration of proof-of-biology. Such substrates of the Gcase enzyme are elevated in the cerebrospinal fluid of Parkinson's disease patients who harbor the GBA1 mutation, and we expect that substrate levels would be normalized if our gene therapy restores Gcase enzyme expression in the brain. This gene therapy may also have potential utility in idiopathic Parkinson's disease, where there is evidence of loss of Gcase activity in the substantia nigra in Parkinson's disease patients even in the absence of GBA1 mutations as well as evidence of lysosomal dysfunction in general.

Preclinical Studies

At the American Society of Gene & Cell Therapy 25th Annual Meeting in May 2022, or the ASGCT 2022 Meeting, we presented preclinical data demonstrating CNS target engagement and delivery of therapeutically relevant levels of Gcase in a GBA1 loss of function mouse model, as well as sustained expression for three or more months following intravenous administration.

Program Status

Under the 2023 Neurocrine Collaboration Agreement, we are developing gene therapy products directed to the gene that encodes GBA1 for the treatment of Parkinson's disease and other diseases associated with GBA1. The GBA1 Program is currently in preclinical development. We and Neurocrine are in the process of identifying a lead candidate that will comprise a TRACER capsid, promoter, and transgene. For more information, refer to Note 15 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Early Research Programs

In January 2023, we announced the launch of an updated early research initiative for the treatment of Huntington's disease. The updated gene therapy program, which leverages the latest insights in disease biology, combines an intravenous TRACER capsid with vectorized siRNAs to enable specific knockdown of mHTT and MSH3.

During the first quarter of 2023, we announced an early research initiative investigating a gene therapy targeting intracellular tau for the treatment of Alzheimer's disease. The program combines an siRNA tau knockdown payload with an intravenously delivered TRACER capsid.

Our wholly-owned early research programs also include a program exploring a vectorized antibody against HER2 for the treatment of brain metastases from metastatic breast cancer. Pre-clinical data has demonstrated that our vectorized antibody against HER2 is shown to inhibit proliferation and promote antibody-dependent cell cytotoxicity, a process that recruits natural killer cells, macrophages and/or brain-resident innate immune cells called microglia to eliminate tumor cells.

Collaborations and License Agreements

Pfizer Option and License Agreement

On October 1, 2021, or the Pfizer Effective Date, we entered into the Pfizer Agreement with Pfizer pursuant to which we granted Pfizer the Pfizer License Options to certain TRACER capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer Transgenes. Under the terms of the Pfizer Agreement, during an initial research term that ended as of October 1, 2022, or the Pfizer Research Term, Pfizer had the right to evaluate the potential use of the capsids in combination with up to two Pfizer Transgenes to help treat respective CNS and cardiovascular diseases.

Research and License Option

During the Pfizer Research Term, we agreed to provide Pfizer with certain quantities of materials encoding specified existing capsids for Pfizer's evaluation. Further, during the Pfizer Research Term, we agreed to disclose to Pfizer, on a rolling basis, the performance characteristics identified during the Pfizer Research Term for all such capsid candidates. Pfizer had the right, in its sole discretion, to select any capsid candidate for evaluation to determine its interest in exercising a Pfizer License Option with respect to such capsid candidate. Pfizer had the right to exercise up to two Pfizer License Options, provided that it could exercise only one Pfizer License Option for each Pfizer Transgene.

Effective as of September 30, 2022, Pfizer exercised a Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to us. Pfizer's exercise of a Pfizer License Option extends the Pfizer Research Term to October 1, 2024, during which period we may, at our sole discretion and expense, conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of the rare neurological disease associated with the exercise of the applicable Pfizer License Option.

In connection with the exercise of the Pfizer License Option for a rare neurological disease, we granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene, or the Pfizer Licensed CNS Products. Until October 1, 2024, while we are not obligated to conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of rare neurological diseases, we have agreed to continue to disclose to Pfizer, on a rolling basis, the performance characteristics identified for all such capsid candidates that we identify during the Pfizer Research Term, if and when available. Pfizer may, during the Pfizer Research Term, conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid it previously elected to license when it exercised the Pfizer License Option.

Development, Regulatory Approval and Commercialization

Under the Pfizer Agreement, Pfizer is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Pfizer Licensed CNS Products. Pfizer is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Pfizer Licensed CNS Product for which Pfizer has exercised its Pfizer License Option in (a) the United States and (b) at least one of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan, each of which is referred to as a Pfizer Major Market Country, subject to certain limitations. Pfizer is also required to use commercially reasonable efforts to commercialize each Pfizer Licensed CNS Product in the United States and at least one Pfizer Major Market Country where Pfizer or its designated affiliates or sublicensees has received regulatory approval for such Pfizer Licensed CNS Product, subject to certain limitations.

Intellectual Property

Under the terms of the Pfizer Agreement, each of us and Pfizer owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the effective date of the Pfizer Agreement, or invented, developed, created, generated or acquired solely by or on behalf of such party after such effective date.

Exclusivity

Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Pfizer Agreement and in the course of our and Pfizer's activities under the Pfizer Agreement will follow inventorship under U.S. patent law. Subject to certain limitations and exceptions, we have agreed (a) during the Pfizer Research Term, not to conduct any internal program or program on behalf of a third party that is directed to development or commercialization of any capsid candidates, or grant any third party or affiliate any right or license under our rights in such capsid candidates to exploit any therapeutic product, in combination with any Pfizer Transgene in any indication for therapeutic, diagnostic and prophylactic human and veterinary use; and (b) after Pfizer's exercise of a Pfizer License Option, not to grant any third party or affiliate any right or license under our patents to exploit any licensed capsid in combination with any Pfizer Transgene.

Financial

Under the terms of the Pfizer Agreement, Pfizer has paid us an upfront payment of $30 million and a payment of $10 million in connection with the exercise of the Pfizer License Option for a rare neurological disease. We are also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115 million for the first corresponding Pfizer Licensed CNS Product to achieve the corresponding milestone. On a Pfizer Licensed CNS Product-by-Pfizer Licensed CNS Product basis, we are also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175 million per Pfizer Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Pfizer Licensed CNS Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Termination

Unless earlier terminated, the Pfizer Agreement expires on the expiration of the last-to-expire royalty term with respect to all Pfizer Licensed CNS Products in all countries. Subject to a cure period, either party may terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Pfizer may also terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, for our insolvency, the occurrence of a violation of global trade control laws, or for our non-compliance with certain anti-bribery or anti-corruption covenants. Pfizer may also terminate the Pfizer Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Pfizer, the license granted by us to Pfizer under the Pfizer Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Pfizer under such license had the Pfizer Agreement remained in effect would be substantially reduced.

Novartis Option and License Agreement

On March 4, 2022, or the Novartis Effective Date, we entered into the Novartis Agreement with our collaborative partner Novartis. Pursuant to the Novartis Agreement, we have granted Novartis the Novartis License Options to license Novartis Licensed Capsids, for exclusive use with certain targets to develop and commercialize a Novartis Payload.

Research and License Option

During the period commencing on the Novartis Effective Date and ending on the first anniversary thereof or, in the event Novartis exercises a Novartis License Option, the third anniversary thereof, we have granted Novartis a non-exclusive research license to evaluate our TRACER capsids for potential use, in combination with Novartis Payloads, in programs targeting three specified genes, or the Initial Novartis Targets. We refer to this period, on a target-by-target basis, as the Novartis Research Term. Upon the payment of additional fees, Novartis may also assess our TRACER capsids for use with up to two other targets, or the Additional Novartis Targets, subject to certain conditions including that such target is not part of, or reasonably competitive with, our current development programs. During the Novartis Research Term, as applicable, we may, at our sole discretion and expense, conduct further research activities to identify additional TRACER capsids. If we elect to do so, we have agreed to disclose performance characteristics of such new TRACER capsids to Novartis on a rolling basis.

During the applicable Novartis Research Terms, Novartis may exercise up to three Novartis License Options—or up to five Novartis License Options if Novartis is evaluating the Additional Novartis Targets—in the aggregate, provided that Novartis may only exercise one Novartis License Option for each Novartis Target. Upon the exercise of any Novartis License Option, we have agreed to grant Novartis a target-exclusive, worldwide license, with the right to sublicense, under certain of our intellectual property, the rights to develop and commercialize the applicable Novartis Licensed Capsid as incorporated into products containing the corresponding Novartis Payload, or the Novartis Licensed Products. Upon the exercise of a Novartis License Option, we have agreed to provide certain additional know-how to enable Novartis to exploit the Novartis Licensed Capsid and the corresponding Novartis Payload for use in a Novartis Licensed Product. Novartis may, during the applicable Novartis Research Term but following the exercise of a Novartis License Option, conduct additional evaluation of our capsid candidates and has the right to substitute any other TRACER capsid for the Novartis Licensed Capsid.

Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from our TRACER capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. With Novartis' option exercise on two Initial Novartis Targets, we are entitled to receive a $25.0 million option exercise payment during the first half of 2023, and we are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in our internal and partnered pipeline. In addition, over the next 18 months, Novartis retains the right to expand the agreement to include options to license capsids for up to two Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and mid- to high-single-digit tiered royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids.

Novartis elected not to license a capsid for one Initial Novartis Target under the Novartis Agreement prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that we granted to Novartis in connection with this Initial Novartis Target has terminated, the Novartis Research Term for this Initial Novartis Target has expired, and we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this Initial Novartis Target. All capsid rights with respect to that Initial Novartis Target have returned to us.

Governance

Subject to our disclosure obligations described above, we and Novartis have agreed to conduct our respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of us and Novartis.

Development, Regulatory Approval and Commercialization

Under the Novartis Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Novartis Licensed Products. In the event Novartis exercises a Novartis License Option, Novartis is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Novartis Licensed Product for each Novartis Target for which it has exercised a Novartis License Option in (a) the United States and (b) at least three of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan, each of which, a Novartis Major Market Country, subject to certain limitations. Novartis is also required to use commercially reasonable efforts to commercialize each Novartis Licensed Product in the United States and at least three Novartis Major Market Countries where Novartis or its designated affiliates or sublicensees has received regulatory approval for such Novartis Licensed Product, subject to certain limitations.

During the applicable Novartis Research Term, we have agreed to provide plasmids to Novartis for the production of TRACER capsids for evaluation upon request. We have also granted Novartis a non-exclusive license, effective upon an exercise of a Novartis License Option and in addition to its options for target-exclusive licenses under certain of our intellectual property described above, on a Novartis Licensed Capsid-by-Novartis Licensed Capsid basis, under certain of our know-how to exploit the applicable Novartis Licensed Capsid as incorporated into Novartis Licensed Products containing the corresponding Novartis Payload.

Financial

Under the terms of the Novartis Agreement, Novartis has paid us an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license two novel capsids generated from our TRACER capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. With the exercise of two Novartis License Options, we have become entitled to receive a $25.0 million option exercise payment during the first half of 2023, and we are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in our internal and partnered pipeline. In addition, over the next 18 months, Novartis retains the right to expand the collaboration to include options to license capsids for up to two Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and mid- to high-single-digit tiered royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Intellectual Property

Under the terms of the Novartis Agreement, each party owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the Novartis Effective Date, or invented, developed, created, generated or acquired solely by or on behalf of such party after the Novartis Effective Date. Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Novartis Agreement and in the course of the parties' activities under the Novartis Agreement will follow inventorship under U.S. patent law.

Exclusivity

Subject to certain limitations and exceptions, we have agreed (a) during the Novartis Research Term, as applicable, not to conduct any internal program or program on behalf of a third party that is directed to the development or commercialization of any our capsids, or grant any third party or affiliate any right or license under our rights in such capsids, to exploit any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on any of the Targets; and (b) after Novartis's exercise of any Novartis License Option, not to grant

any third party or affiliate any right or license under our patents to exploit any Novartis Licensed Capsid for the applicable Target.

Termination

Unless earlier terminated, the Novartis Agreement expires on the expiration of the last-to-expire royalty term with respect to all Novartis Licensed Products in all countries. Subject to a cure period, either party may terminate the Novartis Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the Novartis Agreement, in whole or in part, subject to specified conditions, for our insolvency, the occurrence of a violation of global trade control laws, or for our non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the Novartis Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Novartis, the licenses granted by us to Novartis under the Novartis Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the Novartis Agreement remained in effect would be substantially reduced.

Neurocrine Collaborations

2019 Neurocrine Collaboration Agreement

In January 2019, we entered into the 2019 Neurocrine Collaboration Agreement for the research, development and commercialization of certain of our AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, we agreed to collaborate on the conduct of the four 2019 Neurocrine Programs.

Collaboration and Licenses

Under the terms of the 2019 Neurocrine Collaboration Agreement, subject to the rights retained by us thereunder, we agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of our intellectual property rights, for all human and veterinary diagnostic, prophylactic, and therapeutic uses, for the research, development, and commercialization of gene therapy products, which we refer to as the 2019 Collaboration Products, under (a) the VY-AADC Program, on a worldwide basis; (b) the FA Program, in the United States and, all countries in the world in which the 2019 Neurocrine Collaboration Agreement remains in effect with respect to the FA Program; and (c) each 2019 Discovery Program, on a worldwide basis. Licenses related to the VY-AADC Program terminated in August 2021.

As a result of the June 2019 Sanofi Genzyme Termination Agreement, we gained worldwide rights to the Huntington's disease program for VY-HTT01 and ex-U.S. rights to the FA program. We subsequently transferred the ex-U.S. rights to the FA Program to Neurocrine pursuant to the 2019 Neurocrine Collaboration Agreement. To facilitate our transfer of the ex-U.S. rights to the FA Program to Neurocrine, we and Neurocrine amended the 2019 Neurocrine Collaboration Agreement and we received a $5.0 million payment from Neurocrine.

Pursuant to development plans to be agreed by the parties, which are overseen by a joint steering committee, or JSC, we have operational responsibility, subject to certain exceptions, for the conduct of each 2019 Neurocrine Program prior to the Transition Event for each Program, as described below, and are required to use commercially reasonable efforts to develop the 2019 Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by us in conducting these activities for each 2019 Neurocrine Program, in accordance with an agreed budget. If we breach our development responsibilities or in certain circumstances upon a change in control of us, Neurocrine has the right but not the obligation to assume the activities under such 2019 Neurocrine Program.

Upon the occurrence of a specified event for each 2019 Neurocrine Program, or a 2019 Transition Event, Neurocrine agreed to assume responsibility for development, manufacturing and commercialization activities for such

2019 Neurocrine Program from us and to pay milestones and royalties on future net sales as described further below. For each Legacy Program, we were granted the option, or a 2019 Co-Co Option, to co-develop and co-commercialize such 2019 Neurocrine Program upon the occurrence of a specified event, or a 2019 Co-Co Trigger Event. We agreed, upon our exercise of a 2019 Co-Co Option, to enter into a cost- and profit-sharing arrangement with Neurocrine, or a 2019 Co-Co Agreement, and (a) jointly develop and commercialize 2019 Collaboration Products for such 2019 Neurocrine Program, or 2019 Co-Co Products, (b) share in its costs, profits and losses, and (c) forfeit certain milestones and royalties on net sales in the United States during the effective period of the applicable 2019 Co-Co Agreement. The 2019 Co-Co Option has expired, and the 2019 Transition Event and the 2019 Co-Co Trigger Event are no longer applicable, with respect to the VY-AADC Program in light of the termination of the 2019 Neurocrine Collaboration Agreement with respect to the program. The remaining 2019 Transition Events are (a) with respect to the FA Program, our receipt of topline data for the initial Phase 1 clinical trial for an FA Program product candidate; and (b) with respect to each 2019 Discovery Program, the preparation by us and the approval by Neurocrine of an IND application to be filed with the FDA by Neurocrine for the first development candidate in such 2019 Discovery Program. The 2019 Co-Co Trigger Event for the FA Program is the achievement of milestones or metrics specified in the applicable development plan, as determined by the JSC.

Under the 2019 Neurocrine Collaboration Agreement, subject to exceptions specified, we and Neurocrine agreed that profits and losses under our 2019 Co-Co Option would be allocated (a) 50% to Neurocrine and 50% to us for a 2019 Collaboration Product from the VY-AADC Program and (b) 60% to Neurocrine and 40% to us for a 2019 Collaboration Product from the FA Program; provided, however, that Neurocrine would have the right to elect, within a specified period following the acceptance for filing of a biologics license application, or BLA, from the FDA, to pay a $35.0 million rate-shifting fee to us to change the allocation for the VY-AADC Program to 55% to Neurocrine and 45% to us. The parties agreed that each 2019 Co-Co Agreement would provide us the right to terminate for any reason upon prior written notice to Neurocrine and Neurocrine the right to terminate in certain circumstances upon our change of control.

Governance

Our research and development activities under the 2019 Neurocrine Collaboration Agreement are to be conducted pursuant to plans agreed to by the parties, on a program-by-program basis, and overseen by the JSC, which is composed of an equal number of representatives from the parties. The JSC may delegate matters within its authority to subcommittees of the JSC. In addition, the 2019 Neurocrine Collaboration Agreement establishes working groups to handle specified matters on a subject matter-by-subject matter basis. If a working group or subcommittee cannot agree on a matter within its purview within a specified time, such matter is to be referred sequentially to the JSC and then the executive officers of the parties. If the executive officers are not able to resolve the matter, then (a) with respect to each Legacy Program, subject to specified exceptions, (x) Neurocrine has the right to resolve such matter prior to our exercise of our 2019 Co-Co Option with regard to such 2019 Co-Co Product or if such 2019 Co-Co Option expires or goes unexercised and (y) following the timely exercise by us of our 2019 Co-Co Option, depending on the subject of such matter, either Neurocrine, in certain instances, or the parties jointly or the JSC, in other instances, would have the right to resolve such matter, and (b) with respect to 2019 Discovery Programs, subject to specified exceptions, Neurocrine has the right to resolve such matter.

Candidate Selection

The parties have committed to agree on a list of up to eight target genes, or Targets, from which Neurocrine has the right to nominate Targets for the two 2019 Discovery Programs. The Targets nominated for the 2019 Discovery Programs must be approved by a consensus of the JSC or the executive officers.

Manufacturing

Prior to the 2019 Transition Event for a 2019 Neurocrine Program, we are responsible for the manufacture of any 2019 Collaboration Products for the 2019 Neurocrine Program. Following the Transition Event, the parties shall negotiate the manufacturing and supply responsibilities, subject to the terms of any applicable 2019 Co-Co Agreement.

Financial Terms

Under the terms of the 2019 Neurocrine Collaboration Agreement, Neurocrine has paid us an upfront payment of $115.0 million. In connection with the 2019 Neurocrine Collaboration Agreement, Neurocrine also paid us $50.0 million as consideration for an equity purchase of 4,179,728 shares of our common stock. The 2019 Neurocrine Collaboration Agreement provides for aggregate development milestone payments from Neurocrine to us for 2019 Collaboration Products under (a) the VY-AADC Program of up to $170.0 million, which we are no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) the FA Program of up to $195.0 million, and (c) each of the two 2019 Discovery Programs of up to $130.0 million per 2019 Discovery Program. We may be entitled to receive aggregate commercial milestone payments for each 2019 Collaboration Product of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $1.1 billion.

Neurocrine has also agreed to pay us royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the VY-AADC Program, from the mid-teens to thirty and the low-teens to twenty, respectively, which we are no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (c) for each 2019 Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a 2019 Collaboration Product and terminate on the later of (x) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (y) 10 years from the first commercial sale of the 2019 Collaboration Product in such country and (z) the expiration of regulatory exclusivity in such country, or the 2019 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patents rights related to a 2019 Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any 2019 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the 2019 Royalty Term applicable to such 2019 Collaboration Product in such country.

Intellectual Property

Under the terms of the 2019 Neurocrine Collaboration Agreement and subject to specified exceptions therein, each party owns the entire right, title and interest in and to all intellectual property rights made solely by its employees or agents in the course of the collaboration. The parties jointly own all rights, title and interest in and to all intellectual property rights made or invented jointly by employees or agents of both parties.

Exclusivity

During the term of the 2019 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly exploit any AAV-based gene therapy products directed to a Target to which a 2019 Collaboration Product is directed, subject to specified exceptions, including the parties' conduct of basic research activities.

Termination

Unless earlier terminated, the 2019 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2019 Royalty Term with respect to a 2019 Collaboration Product in all countries in the relevant territory or (b) the expiration or termination of all 2019 Co-Co Agreements. Neurocrine may terminate the 2019 Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least (x) 180-day advance notice if such notice is provided prior to the first commercial sale of the 2019 Collaboration Product to which the termination applies or (y) one-year advance notice if such notice is provided after the first commercial sale of the 2019 Collaboration Product to which the termination applies. We may terminate the 2019 Neurocrine Collaboration Agreement, subject to specified conditions, if Neurocrine challenges the validity or

enforceability of certain of our intellectual property rights. Subject to a cure period, either party may terminate the 2019 Neurocrine Collaboration Agreement in the event of a material breach by the other party in whole or in part, subject to specified conditions.

Upon termination in certain cases, Neurocrine has agreed to grant to us licenses to certain Neurocrine intellectual property, subject to a negotiation between the parties to establish royalty rates for use of such intellectual property. In the event of a breach by us with respect to a 2019 Neurocrine Program, if such termination were to occur after a 2019 Transition Event, then (a) if a 2019 Co-Co Agreement is in effect with respect to such program, Neurocrine can terminate the 2019 Co-Co Agreement for such program and we would no longer have co-development and co-commercialization rights with respect to the 2019 Collaboration Product and (b) subject to any license agreements, Neurocrine would no longer have any obligations with respect to any 2019 Collaboration Products resulting from such program.

On February 2, 2021, Neurocrine notified us that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program, effective as of the Neurocrine VY-AADC Program Termination Effective Date. The 2019 Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. As a result of the termination, as of the Neurocrine VY-AADC Program Termination Effective Date, the license granted by us to Neurocrine thereunder regarding the VY-AADC Program expired and we regained worldwide intellectual property rights regarding the VY-AADC Program.

2023 Neurocrine Collaboration Agreement

On January 8, 2023, we entered into the 2023 Neurocrine Collaboration Agreement for the research, development, manufacture and commercialization of the 2023 Neurocrine Programs.

Collaboration and License

Under the 2023 Neurocrine Collaboration Agreement, we and Neurocrine have agreed to collaborate on the conduct of the 2023 Neurocrine Programs. The 2023 Neurocrine Collaboration Agreement became effective upon the expiration of all applicable waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, which occurred on February 21, 2023, or the Neurocrine Effective Date. Under the terms of the 2023 Neurocrine Collaboration Agreement, subject to the rights retained by us thereunder, we granted to Neurocrine, as of the Neurocrine Effective Date, an exclusive, royalty-bearing, sublicensable, worldwide license, under certain of our intellectual property rights, to research, develop, manufacture and commercialize gene therapy products, or the 2023 Collaboration Products, arising under the 2023 Neurocrine Programs.

Pursuant to mutually-agreed development plans, during the period beginning on the Neurocrine Effective Date and ending on the third anniversary of the Neurocrine Effective Date, which period may be extended upon mutual written agreement of us and Neurocrine, or the 2023 Discovery Period, and as overseen by the JSC that oversees our ongoing collaboration with Neurocrine, we are responsible for identifying capsids meeting target criteria, producing development candidates, and conducting other non-clinical activities regarding the 2023 Collaboration Products. Neurocrine has agreed to be responsible for all costs we incur in conducting non-clinical development activities for each 2023 Neurocrine Program, in accordance with an agreed budget. If we breach our development responsibilities or, in certain circumstances, upon a change of control, Neurocrine has the right, but not the obligation, to assume the conduct of our activities under such 2023 Neurocrine Program.

We have been granted the option, or a 2023 Co-Co Option, to co-develop and co-commercialize 2023 Collaboration Products in the GBA1 Program in the United States upon the occurrence of a specified event, or a 2023 Co-Co Trigger Event. Should we elect to exercise our 2023 Co-Co Option, we and Neurocrine agree to enter into a cost- and profit-sharing arrangement, or a 2023 Co-Co Agreement, whereby we and Neurocrine agree to jointly develop and commercialize 2023 Collaboration Products in the GBA1 Program, or 2023 Co-Co Products, in the United States and share equally in the GBA1 Program's costs, profits and losses in the United States, with each party entitled to or responsible for 50% of profits and losses with respect to each 2023 Co-Co Product in the United States, subject to specified exceptions. The parties have agreed that the 2023 Co-Co Agreement will provide us the right to terminate the

2023 Co-Co Agreement for any reason upon prior written notice to Neurocrine and provide Neurocrine the right to terminate or amend the 2023 Co-Co Agreement upon a change of control under certain circumstances. In the event we exercise our 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50% share of profits) 50% of our share of profits until our obligation to repay 50% of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50% of our share of profits. The 2023 Co-Co Trigger Event is the date on which we receive topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program.

Governance

Our research and development activities under the 2023 Collaboration Agreement are to be conducted pursuant to plans agreed to by the parties, on a 2023 Neurocrine Program-by-2023 Neurocrine Program basis, and overseen by the JSC, which is composed of an equal number of representatives from each of us and Neurocrine. The JSC may delegate matters within its authority to subcommittees of the JSC. In addition, the 2023 Collaboration Agreement establishes working groups to handle specified matters on a subject matter-by-subject matter basis. If a working group or subcommittee cannot agree on a matter within its purview within a specified time, such matter is to be referred sequentially to the JSC and then the executive officers of the parties. If the executive officers are not able to resolve the matter, then (a) with respect to the GBA1 Program, subject to specified exceptions, (x) Neurocrine has the right to resolve such matter prior to our exercise of our 2023 Co-Co Option for the GBA1 Program or in the event we elect not to exercise our 2023 Co-Co Option, and (y) following the exercise by us of our 2023 Co-Co Option for the GBA1 Program, depending on the subject of such matter, either Neurocrine, in certain instances, or the parties jointly or the JSC, in other instances, would have the right to resolve such matter, and (b) with respect to the 2023 Discovery Programs, subject to specified exceptions, Neurocrine has the right to decide any unresolved matters relating to a 2023 Discovery Program that are within the JSC's authority.

Candidate Selection

Either party may notify the JSC of any gene therapy product candidate that includes a Voyager capsid and a payload that is being developed under a 2023 Neurocrine Program, or a Collaboration Candidate, that it desires to nominate as a development candidate. In such event, the JSC shall determine whether such nominated Collaboration Candidate meets certain development criteria. There will be a maximum of four potential development candidates for which development is being performed under any 2023 Neurocrine Program at any given time during the 2023 Discovery Period. If a Collaboration Candidate fails to meet criteria established by the JSC and is removed from consideration to become a development candidate or is named a development candidate, then a new Collaboration Candidate may be nominated to be a potential development candidate to replace the Collaboration Candidate that has failed or succeeded such that not more than four potential development candidates per program are under consideration at any one time during the 2023 Discovery Period.

Manufacturing

The parties have agreed that the applicable development plans shall specify the allocation between us and Neurocrine of responsibilities for the manufacturing of Collaboration Candidates associated with the applicable 2023 Neurocrine Program during the 2023 Discovery Period. In accordance with the 2023 Collaboration Agreement, the parties have also agreed that, if we conduct any portion of the manufacturing of a Collaboration Candidate, the applicable development plan shall include an obligation for us to assist with the technology transfer of such manufacturing responsibilities to Neurocrine or a third-party contract manufacturing organization, as reasonably requested by Neurocrine, on terms to be mutually-agreed by us and Neurocrine. Following the end of the 2023 Discovery Period, Neurocrine shall be responsible for the manufacturing of all Collaboration Candidates and products

Financial Terms

Under the terms of the 2023 Neurocrine Collaboration Agreement, Neurocrine paid us an upfront payment of approximately $136.0 million and approximately $39.0 million as consideration for an equity purchase of 4,395,588 shares of our common stock in February 2023. The 2023 Collaboration Agreement provides for aggregate development

milestone payments from Neurocrine to us for 2023 Collaboration Products under (a) the GBA1 Program of up to $985.0 million; and (b) each of the three 2023 Discovery Programs of up to $175.0 million for each 2023 Discovery Program. We may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $950.0 million per 2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $275.0 million per 2023 Discovery Program.

Neurocrine has also agreed to pay us tiered royalties, based on future net sales of the 2023 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, range from (a) for the GBA1 Program, the low double-digits to twenty and the high single-digits to mid-teens, respectively, and (b) for each 2023 Discovery Program, high single-digits to mid-teens and mid-single digits to low double-digits, respectively. On a country-by-country and 2023 Neurocrine Program-by-2023 Neurocrine Program basis, the parties have agreed royalty payments would commence on the first commercial sale of a 2023 Collaboration Product in such country and terminate upon the latest of (a) the expiration, invalidation or the abandonment of the last patent covering the composition of the 2023 Collaboration Product or its approved method of use in such country, (b) ten years from the first commercial sale of the 2023 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country, or the 2023 Royalty Term. Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patent rights related to a 2023 Collaboration Product, approval of biosimilar products in a given country, or required payment of licensing fees to third parties related to the development and commercialization of any 2023 Collaboration Product. Additionally, the licenses granted to Neurocrine shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and 2023 Collaboration Product-by-2023 Collaboration Product basis upon the expiration of the 2023 Royalty Term applicable to the 2023 Collaboration Product in such country.

Intellectual Property

Under the terms of the 2023 Neurocrine Collaboration Agreement, each party owns all right, title and interest in and to all patent rights or know-how controlled by such party and existing as of or before the Neurocrine Effective Date or created or acquired solely by or on behalf of such party (including through its or its affiliate's representatives) after the Neurocrine Effective Date outside of its activities under the 2023 Neurocrine Collaboration Agreement. The parties have further agreed that all know-how created by either or both parties in the performance of the activities as undertaken pursuant to a development plan during the 2023 Discovery Period or in the course of development, manufacture and commercialization of Collaboration Candidates or products and all patent rights covering such know-how, or collectively the 2023 Arising IP, is to be owned as follows: (a) we solely own all 2023 Arising IP created jointly by representatives of us and Neurocrine that constitutes capsid know-how and capsid patent rights, and 2023 Arising IP created solely by representatives of Neurocrine through the use of our confidential information, including unpublished sequence information for our capsids; and (b) with respect to all other 2023 Arising IP, (x) we solely own all such 2023 Arising IP created solely by its representatives, (y) Neurocrine solely owns all such 2023 Arising IP created solely by its representatives; and (z) the parties jointly own all such 2023 Arising IP created jointly by representatives of both Neurocrine and us. 2023 Arising IP owned by us is included in the license granted from us to Neurocrine described above.

Exclusivity

During the term of the 2023 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly develop, manufacture or commercialize any other gene therapy product directed to a target under any 2023 Neurocrine Program, or grant any affiliate or third-party a license or sublicense to enable any third-party to do so, subject to specified exceptions, including the parties' conduct of certain basic research, provided that Neurocrine or its affiliates may develop competitive products that do not contain an adeno-associated virus as the viral vector.

Termination

Unless earlier terminated, the 2023 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2023 Royalty Term with respect to all 2023 Collaboration Products worldwide or (b) the expiration or termination of any 2023 Co-Co Agreement. Neurocrine may terminate the 2023 Neurocrine Collaboration

Agreement in its entirety or on a 2023 Neurocrine Program-by-2023 Neurocrine Program and/or country-by-country basis by providing at least (a) 180-day advance notice if such notice is provided prior to the first commercial sale of any 2023 Collaboration Product to which the termination applies or (b) one-year advance notice if such notice is provided after the first commercial sale of any product to which the termination applies. Neurocrine may terminate the 2023 Neurocrine Collaboration Agreement with respect to a given 2023 Collaboration Product by providing written notice of termination to us within thirty days after complete readout of any clinical trial if the results of such clinical trial fail to meet the pre-specified primary endpoint(s) set forth in the applicable protocol or if there is a safety finding during the clinical trial relating to such 2023 Collaboration Product that either (a) is substantially irreversible or not monitorable in patients or (b) results in Neurocrine's decision to designate such 2023 Collaboration Product as a terminated product under the 2023 Collaboration Agreement.

We may terminate the 2023 Neurocrine Collaboration Agreement with respect to a particular patent right of ours, if Neurocrine challenges the validity or enforceability of such patent right. Subject to a cure period, either party may terminate the 2023 Neurocrine Collaboration Agreement in the event of a material breach in whole or in part, subject to specified conditions.

2023 Neurocrine Stock Purchase Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, Neurocrine and the Company also entered into a stock purchase agreement on the January 8, 2023 for the sale and issuance of 4,395,588 shares of common stock to Neurocrine at a price of $8.88 per share, for an aggregate purchase price of approximately $39.0 million. In accordance with the terms and conditions of the stock purchase agreement, we issued and sold these shares to Neurocrine on February 23, 2023.

2023 Neurocrine Amended and Restated Investors Rights Agreement

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, Neurocrine and the Company also amended and restated their existing investor agreement on January 8, 2023, or the 2023 Neurocrine Amended and Restated Investor Agreement, providing for standstill and lock-up restrictions and a voting agreement with respect to shares of the Company owned by Neurocrine. Pursuant to the 2023 Neurocrine Amended and Restated Investor Agreement, the Company caused Jude Onyia, Ph.D., Chief Scientific Officer of Neurocrine, to be appointed to the Company's board of directors as a Class III director on February 23, 2023. The Company has agreed that it shall cause Dr. Onyia, or another individual designated by Neurocrine, to be nominated for election to the Company's board of directors when Dr. Onyia's initial term is scheduled to expire. Under the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine's right to designate an individual to serve as a director on the Company's board of directors and the Company's agreement to nominate such individual for election to the Company's board of directors is subject to specified conditions and shall terminate upon the earliest of (a) Neurocrine holding less than 10% of the Company's outstanding common stock; (b) a change of control of the Company or Neurocrine; (c) a liquidation or dissolution of the Company; and (iv) the date that is ten years from the closing date of the 2023 Neurocrine Amended and Restated Investor Agreement.

Pursuant to the terms of the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine has agreed not to, without the prior written approval of the Company and subject to specified conditions, directly or indirectly acquire shares of the Company's outstanding common stock, seek or propose a tender or exchange offer or merger between the parties, solicit proxies or consents with respect to any matter, or undertake other specified actions related to the potential acquisition of additional equity interests in the Company (the "Standstill Restrictions"). Further, Neurocrine has also agreed not to, and to cause its affiliates not to, sell or transfer any shares of the Company without the prior written approval of the Company, subject to specified conditions (the "Lock-Up Restrictions").

In addition, pursuant to the terms of the 2023 Neurocrine Amended and Restated Investor Agreement, Neurocrine has agreed that any shares of the Company it owns are subject to a voting agreement such that, subject to specified conditions and excluding specified extraordinary matters, Neurocrine has agreed to, and has agreed to cause its permitted transferees to, vote in accordance with the recommendation of the Company's board of directors and has granted the Company an irrevocable proxy with respect to the foregoing (the "Voting Agreement").

Each of the Standstill Restrictions, the Lock-Up Restrictions, and the Voting Agreement terminate upon the earliest to occur of: (i) the date that is the third anniversary of the effective date of the 2023 Neurocrine Amended and Restated Investor Agreement and (ii) a liquidation or dissolution of the Company. The Standstill Restrictions and Lock-Up Restrictions also terminate upon the deregistration of the Company's common stock, if earlier. The Lock-Up Restrictions and Voting Agreement also terminate on a change of control of the Company or the date on which Neurocrine and its affiliates beneficially own less than three percent of the common stock of the Company on an outstanding basis. The Standstill Restrictions and Voting Agreement also terminate upon the later of (x) the expiration or termination of the 2019 Neurocrine Collaboration Agreement and (y) the expiration or termination of the 2023 Neurocrine Collaboration Agreement.

License Agreement with Touchlight IP Limited

In November 2022, we and Touchlight entered into the Touchlight License Agreement, to authorize historical use by us of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER capsids created with the use of the Subject DNA Preparation Process.

The terms of the Touchlight License Agreement include a one-time, non-refundable technology access fee of $5.0 million, paid to Touchlight during the fourth quarter of 2022.

The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight by us if we or our program collaborators or licensees choose to utilize in a therapeutic product TRACER capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, we are obligated to pay low single-digit royalties to Touchlight on future payments we receive in connection with licensing of TRACER capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any of our therapeutic programs.

Competition

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our gene therapy platform, product programs, product candidates and scientific expertise in the fields of gene therapy and neuroscience provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

We are aware of several companies focused on developing AAV gene therapies in various indications, including AavantiBio, Inc. (acquired by Solid Biosciences, Inc., or Solid), Abeona Therapeutics, Inc., Adverum Biotechnologies, Inc., Aevitas Therapeutics, Inc., Akuous, Inc. (acquired by Eli Lilly and Company, or Eli Lilly), Alcyone Therapeutics, Inc., Amicus Therapeutics, Inc., Apic Bio, Inc., Applied Genetic Technologies Corporation (acquired by Syncona Limited), Asklepios BioPharmaceutical, Inc., or AskBio (acquired by Bayer), Audentes Therapeutics, Inc. (acquired by Astellas Pharma Inc.), Biogen, Inc., or Biogen, Brain Neurotherapy Bio, Inc. (merged with AskBio), BioMarin Pharmaceuticals, Inc., Encoded Therapeutics, Inc., GenSight Biologics SA, Homology Medicines, Inc., LEXEO Therapeutics, Inc., LogicBio Therapeutics, Inc. (acquired by AstraZeneca), Lysogene SA, MeiraGTx Ltd., or MeiraGTx, Neurogene, Inc., Novartis Gene Therapies, Inc. (formerly AveXis, Inc.), Passage Bio, Inc., Pfizer, Inc., Prevail Therapeutics, Inc. (acquired by Eli Lilly), PTC Therapeutics, Inc., or PTC, REGENXBio Inc., Sarepta Therapeutics, Inc., Solid, Spark Therapeutics, Inc. (acquired by Roche), StrideBio, Inc., Taysha Gene Therapies, Inc. and uniQure, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

We expect that our TRACER discovery platform and preclinical programs will compete with a variety of therapies in development, including:

- Our TRACER discovery platform will potentially compete with a variety of companies developing AAV capsids, including: 4D Molecular Therapeutics, Inc., Affinia Therapeutics Inc., Apertura Gene Therapy, LLC, Capsida Biotherapeutics, Inc., Capsigen Inc., Dyno Therapeutics, Inc., Kate Therapeutics, Inc., Shape Therapeutics Inc., and StrideBio, Inc.;

- Our program for diseases linked to GBA1 mutations will potentially compete with AAV gene therapies being developed by Prevail Therapeutics Inc. (acquired by Eli Lilly), Freeline Therapeutics Holdings plc, Pfizer, Biogen, Lysogene SA, and Coave Therapeutics SA;

- Our program for tauopathies including AD, progressive supranuclear palsy, and frontotemporal dementia will potentially compete with tau antibodies being developed by Roche Genentech Inc. in collaboration with AC Immune SA, Eli Lilly, AbbVie Biotechnology Ltd, AbbVie Ireland Unlimited Company, Biogen, Eisai Co., Ltd., Janssen Pharmaceuticals, Inc., UCB S.A., and several other companies, as well as an antisense oligonucleotide program being developed by Ionis in collaboration with Biogen;

- Our program for a monogenic form of ALS will potentially compete with Tofersen being developed by Biogen, in collaboration with Ionis, and gene therapies being developed by Novartis Gene Therapies, Inc. and Apic Bio, Inc.; and

- Our FA Program will potentially compete with AAV gene therapies being developed by LEXEO Therapeutics, Inc., AavantiBio, Inc. (acquired by Solid), PTC, StrideBio, Inc. in collaboration with Takeda Pharmaceutical Company Limited, Pfizer, and Novartis Gene Therapies, Inc.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of product candidates and commercializing those product candidates. Accordingly, our competitors may be more successful than us in obtaining approval for product candidates and achieving widespread market acceptance. Our competitors' product candidates may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our product candidates.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new product candidates enter the market and advanced technologies become available. We expect any product candidates that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, and the availability of reimbursement from government and other third-party payers.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Manufacturing

The manufacture of gene therapy products is technically complex, and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. To meet the requirements of our current and planned future trials we have developed a proprietary manufacturing platform that provides a robust and scalable process for AAV production. We are using HEK 293 cell manufacturing to support our preclinical research activities. We also have expertise with the baculovirus/Sf9 AAV production system, a technology for producing AAV vectors at scale in insect-derived cells, which we have used for our clinical development activities in the past and may use in the future for clinical development activities. We focus on developing internal processes and capabilities to produce high-yield and high-quality gene therapies. Both the HEK 293 cell manufacturing process and the baculovirus/Sf9 manufacturing process have been successfully transferred to our contract manufacturing organizations. The baculovirus/Sf9 manufacturing process has been used by our contract manufacturing organizations in manufacturing of clinical materials in accordance with the FDA's current good manufacturing practices, or cGMPs. We have also built an onsite, state-of-the-art process research and development facility to enable the manufacturing of high quality AAV gene therapy vectors at research scale.

We presently contract with third parties for the manufacturing of our program materials. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. The use of contracted manufacturing and reliance on collaboration partners is relatively cost-efficient and we believe that it eliminates the need for our direct investment in manufacturing facilities and additional staff early in development. Although we expect to rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

Intellectual Property

Overview

We strive to protect the proprietary technology, inventions, and know-how to enhance improvements that are commercially important to the development of our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological innovation and on in-licensing opportunities to develop, improve and maintain the strength of our position in the field of gene therapy that may be important for the development of our business. We additionally may rely on regulatory protection afforded through data exclusivity, market exclusivity and patent term extensions where available.

Our commercial success may depend in part on our ability to: obtain and maintain patent and other protections for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and intellectual property rights of third parties. Our ability to stop third parties from making, having made, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable licenses, patents or trade secrets that cover these activities. In some cases, these rights may need to be enforced by third-party licensors. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of manufacturing the same.

We own at least 390 pending patent applications and at least 54 patents have issued in the United States and foreign jurisdictions. We co-own at least 43 pending patent applications and at least 10 patents have issued from these co-owned families in the United States and foreign jurisdictions. At least 12 patent applications have been filed and are pending in the United States and foreign jurisdictions by or on behalf of universities which have granted us exclusive license rights to the technology. To date, at least 44 patents have issued to our licensors which have granted us exclusive license rights to the technology. To date, at least 148 patents have issued to our licensors which have granted us non-exclusive license rights to the technology with at least 68 applications pending. Our policy is to file patent applications to protect technology, inventions and improvements to inventions that are commercially important to the development of our business. We seek United States and international patent protection for a variety of technologies, including: AAV-

based biological products and constructs, methods of delivering said AAV-based biological products and constructs, methods of treating diseases of interest, as well as methods of engineering and manufacturing of the same. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets and that may be used to identify and develop novel biological products. We seek protection, in part, through confidentiality and proprietary information agreements. We are a party to various other license agreements that give us rights to use specific technologies in our research and development.

Company-Owned Intellectual Property

Parkinson's Disease

We own three pending patent families with four issued patents and 35 patent applications directed to AAV constructs encoding the gene AADC for therapeutic uses. Patents that grant from these patent families are generally expected to commence expiration in 2035, subject to possible patent term extensions.

Huntington's Disease

We own five pending patent families with three issued patents and 29 patent applications directed to pharmaceutical compositions and methods for targeting HTT for the treatment of Huntington's disease. Patents from this family are generally expected to commence expiration in 2037, with some applications expiring in 2038, 2040, and 2044 all of which are subject to possible patent term extensions.

ALS

We own five pending patent families and have nine issued patents and 34 patent applications directed to targeting SOD1 for the treatment of ALS. We co-own a sixth patent family with eight pending patent applications directed to pharmaceutical compositions and methods for the treatment of ALS to protect our intellectual property arising from a funded grant from The Amyotrophic Lateral Sclerosis Association. We own one pending patent family with one patent application directed to chromosome 9 open reading frame 72, or C9orf72, for the treatment of ALS. Patents that grant from these patent families are generally expected to commence expiration in 2035, with some applications expiring in 2038, 2039, 2040, and 2042, all of which are subject to possible patent term extensions.

Friedreich's Ataxia

We own three pending patent families with 23 patent applications and we co-own one pending patent family with eight patent applications directed to AAVs encoding frataxin constructs for the treatment of Friedreich's ataxia. Patents that grant from these patent families are generally expected to commence expiration in 2036, with some later filed applications commencing expiration in 2038, 2039, and 2040, all of which are subject to possible patent term extensions.

GBA1 Gene Therapy

We own two pending patent families with 14 pending patent applications directed to AAVs encoding GBA1 for the treatment of Parkinson's disease, Gaucher disease, and dementia with Lewy Bodies. Patents that grant from this patent family are expected to commence expiration in 2041 and 2043, subject to possible patent term extensions.

Vectorized Antibodies

We own four patent families with two issued patents and eight pending patent applications directed to vectorized antibodies and related platforms. Patents that grant from these patent families are generally expected to commence expiration in 2037, with some later filed applications commencing expiration in 2040, all of which are subject to possible patent term extensions.

Tauopathies

We own seven pending patent families directed to antibodies to tau and vectorized forms thereof with 26 pending patent applications. Patents that grant from these families are generally expected to commence expiration in 2037, with some later filed applications commencing expiration in 2040, 2041, 2042, and 2043 all of which are subject to possible patent term extensions. We own one pending patent family to RNA inhibitors for treating tauopathies. Patents that grant from this family are generally expected to commence expiration in 2043, subject to possible patent term extensions.

We have one pending patent family with one pending patent applications directed to pharmaceutical compositions and methods for the treatment of AD. Patents that grant from this family are generally expected to commence expiration in 2043, subject to possible patent term extensions.

Vectorized anti-HER2

We own one pending patent family with one pending patent application directed to AAVs encoding HER2 antibodies for treating metastatic HER2 positive cancers. Patents that grant from these patent families are generally expected to commence expiration in 2042, subject to possible patent term extensions.

Regulatable Expression

We own one pending patent family with three pending patent applications directed to regulatable expression control of AAV transgenes. Patents that grant from this patent family are generally expected to commence expiration in 2036, subject to possible patent term extensions.

Delivery

We own one pending patent family with one patent application directed to cannula delivery system and methods of use. Patents that grant from this patent family are generally expected to commence expiration in 2039, subject to possible patent term extensions.

We co-own two pending patent families directed to trajectory array delivery devices, including the variable trajectory array guide, or V-TAG®, device and methods of use. The first pending patent family has one granted patent and six pending patent applications, and the second pending patent family has one granted patent and six pending patent applications. Patents that grant from these patent families are generally expected to commence expiration in 2037 and 2038, subject to possible patent term extensions.

Capsids

We own two patent families pending in the United States and foreign jurisdictions that are directed to the TRACER discovery platform for selection of AAV capsids with BBB crossing and cell-specific transduction properties. In these two pending patent families directed to the TRACER discovery platform, there are 10 applications pending, and are generally expected to commence expiration in 2039 and 2041, respectively, subject to possible patent term extensions. We also own four pending patent families comprising 34 non-provisional, United States and foreign applications, as well as three pending provisional applications directed to capsid variants identified using the TRACER discovery platform showing improved properties over AAV9. Patents that grant from these patent families and pending provisional applications are generally expected to commence expiration in 2041, 2042 and 2043, subject to possible patent term extensions. We own two pending provisional applications and three pending non-provisional applications directed to constructs containing TRACER capsids in combination with specific payloads for treatment of CNS and other indications. Patents that grant from these pending provisional and non-provisional applications are generally expected to commence expiration in 2042 and 2043, subject to possible patent term extensions.

We also own five patent families pending in the United States and foreign jurisdictions directed to capsid variants generated using other methodologies. In these five pending patent families, there are two granted patents and 20

pending patent applications. Patents that grant from these patent families are generally expected to commence expiration in 2038, subject to possible patent term extensions. We also co-own three patent families directed to other capsid variants. In these three pending patent families there are five pending applications. Patents that grant from these patent families are generally expected to commence expiration in 2039 and 2040, subject to possible patent term extensions.

Vector and Genome Engineering

We own three patent families with 32 issued patents (including 15 patents in European countries) and 45 patent applications directed to engineering of the vector genome. Patents that grant from these patent families are generally expected to commence expiration in 2035, 2037, and 2038, which are all subject to possible patent term extensions.

We own one patent family with one patent application directed to genome engineering. Patents that grant from this patent family are generally expected to commence expiration in 2040, subject to possible patent term extensions.

Production; Chemistry, Manufacturing, and Controls

We own 21 pending patent families with two granted patents and 83 pending patent applications directed to AAV production and CMC. Patents that grant from the earliest filed patent families are generally expected to commence expiration in 2035 and patents that grant from the latest filed patent families are generally expected to commence expiration in 2042, all of which are subject to possible patent term extensions. We co-own one pending patent family with eight granted patent and 11 pending patent applications directed to AAV production and CMC. Patents that grant from this patent family are generally expected to commence expiration in 2037, subject to possible patent term extensions.

Licensed Intellectual Property

We have obtained exclusive licenses and non-exclusive licenses to patents directed to both compositions of matter and methods of use.

We have licensed six families of patents and patent applications, in the field of gene therapy for human diseases, directed to RNAi constructs as vector payloads, their design and use in the treatment of neurological disorders from the University of Massachusetts. Three of the six families of patents and applications are exclusively licensed and comprise 14 granted patents and seven applications in the United States and other territories. Three of the six families of patents and applications are non-exclusively licensed, and comprise 55 granted patents and two applications in the United States and other territories. Patents from these six families have been granted in the United States, Canada, Europe, Israel, Japan, Korea and Australia. Nationalization for some members has taken place in Germany, Spain, France, Great Britain, Italy, and Netherlands. Patents that grant from these patent families are generally expected to expire between 2024 and 2036, subject to possible patent term extensions.

We have exclusively licensed 1 family of patents and patent applications directed to AAV capsids from the University of Massachusetts. In this pending patent family, there are 30 granted patents and six pending patent applications. Patents that grant from this patent family are generally expected to commence expiration in 2030, subject to possible patent term extensions.

We have non-exclusively licensed two pending patent families from Ablexis, LLC. These families of patents and patent applications are pending and/or granted in the United States and other territories and comprise 50 granted patents and 6 applications. Patents have been granted in Australia, Canada, Europe, Korea, New Zealand and the United States. Nationalization for some members has taken place in Austria, Belgium, Denmark, France, Germany, Ireland, Italy, Netherlands, Poland, Spain, Switzerland, and United Kingdom. Patents that grant from these patent families are generally expected to expire between 2029 and 2031, subject to possible patent term extensions.

We have non-exclusively licensed two pending patent families directed to AAV capsids from the California Institute of Technology. These families of patents and patent applications are pending in the United States and internationally and comprise 43 granted patents and 15 applications. Patents have been granted in the United States.

Patents that grant from these patent families are generally expected to commence expiration in 2034 and 2036, subject to possible patent term extensions.

We have non-exclusively licensed three pending patent families directed to microRNA detargeting from the University of Pennsylvania. These families of patent applications are pending in the United States and internationally and comprise 45 applications. Patents that grant from these patent families are generally expected to commence expiration in 2039, 2041, and 2042, subject to possible patent term extensions.

Trademark Protection

We own trademark registrations in the United States for the marks VOYAGER THERAPEUTICS and VOYAGER THERAPEUTICS Logo for "pharmaceutical research and development in the field of gene therapy." We also own pending applications for VOYAGER, and VOYAGER with design elements in the United States, and registrations for VOYAGER with design elements in the European Union and United Kingdom, for goods and services including, among others, "biological preparations for gene therapy," "pharmaceutical research and development in the field of gene therapy," and "medical services provided for clinical trials."

We also own U.S. trademark registrations for the mark V-TAG and the V-TAG Logo, for "medical system comprised of a surgical device for guiding, locating or placing a diagnostic device or therapeutic device, namely, stents, probes, needles, leads, grafts, pumps, syringes, catheters, and implants during a medical procedure and related software sold as a unit, none of the aforesaid for use in cardiac ablation; MRI-compatible medical system comprised of an MRI-compatible surgical device for guiding, locating or placing a diagnostic device or therapeutic device, namely, stents, probes, needles, leads, grafts, pumps, syringes, catheters, and implants during a MRI-guided procedure and related software sold as a unit, none of the aforesaid for use in cardiac ablation," as well as trademark registrations in the European Union and United Kingdom for V-TAG for similar trademark classes.

We also own pending applications in the U.S. and European Union, and a registration in the United Kingdom, for the mark TRACER for services including, among others, "research and development of platform technologies for genetic delivery of therapies and pharmaceutical via adeno-associated virus (AAV) capsids."

We plan to register trademarks in connection with our biological products.

Trade Secret Protection

Finally, we may rely, in some circumstances, on trade secrets to protect our technology. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Government Regulation

The research, development, testing, manufacture, quality control, packaging, labeling, storage, record-keeping, distribution, import, export, promotion, advertising, marketing, sale, pricing and reimbursement of biologic products are extensively regulated by governmental authorities in the United States and other countries. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with compliance with applicable statutes and regulations and other regulatory requirements, both pre-approval and post-approval, require the expenditure of substantial time and financial resources. The regulatory requirements applicable to biological product development, approval and marketing are subject to change, and regulations and administrative guidance often are revised or reinterpreted by the agencies in ways that may have a significant impact on our business.

U.S. Government Regulation

U.S. Biological Products Development Process

In the United States, the FDA approves and regulates gene therapy products as biological products, or biologics. These products are licensed for marketing under the Public Health Service Act, or the PHSA, and regulated under the Federal Food, Drug, and Cosmetic Act, or FDCA. A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products, and for their regulatory approval, is typically referred to as a sponsor.

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to the FDA's good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- preparation of clinical trial material in accordance with cGMPs;
- design of a clinical protocol and submission to the FDA of an application for an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, or ethics committee representing each clinical trial site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety, purity, potency, and efficacy, of the proposed biological product for its intended use;
- submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies, including payment of application user fees;
- satisfactory completion of an FDA inspection prior to BLA approval of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity;
- potential FDA inspection of the nonclinical and clinical study sites that generated the data in support of the BLA;
- potential FDA Advisory Committee meeting to elicit expert input on critical issues and including a vote by external Committee members;
- FDA review and approval, or licensure, of the BLA; and
- compliance with any post approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post approval studies.

Preclinical Studies

Before a sponsor begins testing a product candidate with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry,

formulation and stability, as well as other studies to evaluate, among other things, the toxicity of the product candidate. These studies are generally referred to as IND-enabling studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements, including GLP regulations and standards and the United States Department of Agriculture's Animal Welfare Act, if applicable. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND and are typically referred to as IND-enabling studies. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, and long-term toxicity studies, may continue after the IND is submitted.

The IND and IRB Processes

An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. An IND must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved NDA or BLA. In support of a request for an IND, sponsors must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial may proceed. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a partial clinical hold might state that a specific protocol or part of a protocol may not proceed, while other parts of a protocol or other protocols may do so. No more than 30 days after the imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following the issuance of a clinical hold or partial clinical hold, a clinical investigation may only resume once the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed or recommence. Occasionally, clinical holds are imposed due to manufacturing issues that may present safety issues for the clinical study subjects.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived by the FDA. When a foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. Specifically, the studies must be conducted in accordance with GCP, including undergoing review and receiving approval by an independent ethics committee and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data.

In addition to the foregoing IND requirements, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. The IRB, which must operate in compliance with FDA regulations, must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects and must monitor the trial until completed. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board, or DSMB. This group provides authorization as to whether or not a trial may move forward at designated checkpoints based on review of available data from the study, to which only the

DSMB maintains access. Suspension or termination of development during any phase of a clinical trial can occur if the DSMB determines that the participants or patients are being exposed to an unacceptable health risk.

Human Clinical Trials

Clinical trials involve the administration of the investigational product candidate to human subjects under the supervision of a qualified investigator in accordance with GCP requirements which include, among other things, the requirement that all research subjects provide their informed consent in writing before they participate in any clinical trial. Clinical trials are conducted under written clinical trial protocols detailing, among other things, the objectives of the study, inclusion and exclusion criteria, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol, and any subsequent material amendment to the protocol, must be submitted to the FDA as part of the IND, and progress reports detailing the status of the clinical trials must be submitted to the FDA annually.

Human clinical trials are typically conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may also be required after approval.

Phase 1 clinical trials are initially conducted in a limited population, which may be healthy volunteers or subjects with the target disease, to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics in healthy humans or in patients. During Phase 1 clinical trials, information about the product candidate's pharmacokinetics and pharmacological effects may be obtained to permit the design of well-controlled and scientifically valid Phase 2 clinical trials.

Phase 2 clinical trials are generally conducted in a limited patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials. Phase 2 clinical trials are typically well-controlled and closely monitored.

Phase 3 clinical trials proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are undertaken using a larger patient population to further evaluate dosage, provide substantial evidence of clinical efficacy and further test for safety in an expanded and diverse patient population at multiple geographically dispersed clinical trial sites. A well-controlled, statistically robust Phase 3 clinical trial may be designed to deliver the data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a new biologic product. Such Phase 3 clinical trials are referred to as "pivotal" trials.

A clinical trial may combine the elements of more than one phase and the FDA often requires more than one Phase 3 trial to support marketing approval of a product candidate. A company's designation of a clinical trial as being of a particular phase is not necessarily indicative that the study will be sufficient to satisfy the FDA requirements of that phase because this determination cannot be made until the protocol and data have been submitted to and reviewed by the FDA. Moreover, as noted above, a pivotal trial is a clinical trial that is believed to satisfy FDA requirements for the evaluation of a product candidate's safety and efficacy such that it can be used, alone or with other pivotal or non-pivotal trials, to support regulatory approval. Generally, pivotal trials are Phase 3 trials, but they may be Phase 2 trials if the design provides a well-controlled and reliable assessment of clinical benefit, particularly in an area of unmet medical need.

In December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, actions plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In addition to these requirements, the legislation directs the FDA to issue new guidance on diversity action plans.

In some cases, the FDA may approve an NDA or BLA for a product candidate but require the sponsor to conduct additional clinical trials to further assess the product candidate's safety and effectiveness after approval. Such post-approval trials, typically referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of a larger number of patients in the intended treatment group. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials, such as to verify clinical benefit in the case of products approved under accelerated approval regulations. Failure to exhibit due diligence with regard to conducting mandatory Phase 4 clinical trials could result in withdrawal of FDA approval for products.

Finally, sponsors of clinical trials are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the U.S. National Institutes of Health, or NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. The NIH's Final Rule on registration and reporting requirements for clinical trials became effective in 2017. Although the FDA has historically not enforced these reporting requirements due to a long delay by the Department of Health and Human Services, or HHS, in issuing final implementing regulations, the FDA has issued several Notices of Noncompliance to manufacturers since April 2021. The failure to submit clinical trial information to clinicaltrials.gov, as required, is a prohibited act under the FDCA with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues.

Gene Therapy Products

We expect that the procedures and standards applied to gene therapy products will be applied to any product candidates we may develop. The FDA has defined a gene therapy product as one that seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient. Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Tissues and Advanced Therapies, or OTAT, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The NIH, including the NExTRAC also advises the FDA on gene therapy issues and other issues related to emerging biotechnologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

The FDA has issued various guidance documents regarding gene therapies, including final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, long-term follow-up after the administration of gene therapy products, gene therapies for rare diseases and gene therapies for retinal disorders, as well as draft guidance in January 2021 for Human Gene Therapy for Neurodegenerative Diseases. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, compliance with them is likely necessary to gain approval for any gene therapy product candidate. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the chemistry, manufacturing and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe for potential delayed adverse effects in participants who have received investigational gene therapies with the duration of follow-up based on the potential for risk of such effects. For AAV vectors specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a five-year period.

Until 2019, most gene therapy clinical trials in the United States required pre-review by the predecessor of NExTRAC before being approved by the IRBs and any local biosafety boards or being allowed to proceed by the FDA. In 2019, the NIH substantially eliminated the pre-review process and going forward, the review of gene therapy clinical trial protocols would be largely handled by local IRBs and institutional biosafety committees, or IBCs, in addition to the FDA. Furthermore, in 2019, the NIH removed from public access the Genetic Modification Clinical Research Information System database, which previously contained substantial amounts of safety and other participant information regarding human gene therapy trials performed up to that time.

Manufacturing and Other Regulatory Requirements

Concurrently with clinical trials, sponsors usually complete additional animal safety studies, develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing commercial quantities of the product candidate in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other criteria, the sponsor must develop methods for testing the identity, strength, quality, and purity of the finished product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Specifically, the FDA's regulations require that pharmaceutical products be manufactured in specific approved facilities and in accordance with cGMPs. The cGMP regulations include requirements relating to organization of personnel, buildings and facilities, equipment, control of components and product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports and returned or salvaged products. Manufacturers and other entities involved in the manufacture and distribution of approved pharmaceuticals are required to register their establishments with the FDA and some state agencies, and they are subject to periodic unannounced inspections by the FDA for compliance with cGMPs and other requirements. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside of the United States prior to being imported or offered for import into the United States. Inspections must follow a "risk-based schedule" that may result in certain establishments being inspected more frequently. Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated. Changes to the manufacturing process, specifications or container closure system for an approved product are strictly regulated and often require prior FDA approval before being implemented. The FDA's regulations also require, among other things, the investigation and correction of any deviations from cGMP and the imposition of reporting and documentation requirements upon the sponsor and any third-party manufacturers involved in producing the approved product.

Submission of a BLA to the FDA

FDA approval is required before any new gene therapy product or dosage form, including a new use of a previously approved gene therapy product, can be marketed in the United States. Thus, assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational gene therapy product information is submitted to the FDA in the form of a BLA requesting approval to market the product for one or more indications. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. Under the Prescription Drug User Fee Act, or PDUFA, each BLA must be accompanied by a significant user fee unless an exception or waiver applies, such as the first application filed by a small business or BLAs for product candidates designated as orphan drugs, unless the product candidate includes an indication that is not for a rare disease or condition.

The FDA conducts a preliminary review of all applications within 60 days of receipt and must inform the sponsor at that time or before whether an application is sufficiently complete to permit substantive review. In pertinent part, FDA's regulations state that an application "shall not be considered as filed until all pertinent information and data have been received" by the FDA. In the event that FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. In this event, the BLA must be resubmitted.

If the submission is accepted for filing, the FDA's goal is to review the BLA, within ten months for a standard review, or, if the BLA relates to an unmet medical need in the treatment of a serious or life-threatening condition, perform a priority review, within six months. The review process may be extended by the FDA for three additional months to consider new information or in the case of a clarification provided by the sponsor to address an outstanding deficiency identified by the FDA following the original submission. Despite these review goals, it is not uncommon for FDA review of an application to extend beyond the PDUFA target action date.

The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a REMS is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

In connection with its review of a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, the FDA will typically inspect one or more clinical trial sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements. With passage of FDORA, Congress clarified the FDA's authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to the FDA as well as other persons holding study records or involved in the study process. To assure cGMP and GCP compliance, a sponsor must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

The FDA's Decision on a BLA

The FDA reviews an application to determine, among other things, whether the product is safe, pure and potent for its intended use(s), with the latter determination being made on the basis of substantial evidence. The FDA has interpreted this evidentiary standard to require at least two adequate and well-controlled clinical investigations to establish the efficacy of a new product. Under certain circumstances, however, FDA has indicated that a single trial with certain characteristics and additional information may satisfy this standard. After evaluating the application and all related information, including the advisory committee recommendations, if any, and inspection reports of manufacturing facilities and clinical trial sites, the FDA will issue either a Complete Response Letter, or CRL, or an approval letter. To reach this determination, the FDA must determine that the expected benefits of the proposed product outweigh its potential risks to patients.

A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The CRL may require additional clinical or other data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time- consuming requirements related to clinical trials, preclinical studies or manufacturing. If a CRL is issued, the sponsor will have one year to respond to the deficiencies identified by the FDA, at which time the FDA can deem the application withdrawn or, in its discretion, grant the sponsor an additional six-month extension to respond. For those seeking to challenge the FDA's CRL decision, the FDA has indicated that sponsors may request a formal hearing on the CRL or they may file a request for reconsideration or a request for a formal dispute resolution.

An approval letter, on the other hand, authorizes commercial marketing of the product with specific prescribing information for specific indications. That is, the approval will be limited to the conditions of use (e.g., patient population, indication) described in the FDA-approved labeling. Further, depending on the specific risk(s) to be addressed, the FDA may require that contraindications, warnings or precautions be included in the product labeling, require that post-approval trials, including Phase 4 clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing trials or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Gene therapy products manufactured or distributed pursuant to regulatory approvals are subject to pervasive and continuing regulation by the regulatory authorities, including, among other things, requirements relating to formal commitments for post approval clinical trials and studies, manufacturing, recordkeeping, periodic reporting, product sampling and distribution, marketing, labeling, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior regulatory authority review and approval.

Manufacturers are subject to periodic unannounced inspections by regulatory authorities and country or state agencies for compliance with cGMP and other requirements. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior regulatory approval before being implemented. Regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

Further, although physicians may prescribe legally available products for unapproved uses or patient populations, which are commonly referred to as "off-label uses," manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. In September 2021, the FDA published final regulations which describe the types of evidence that the FDA will consider in determining the intended use of a biologic. If a company is found to have promoted off-label uses, it may become subject to administrative and judicial enforcement by the FDA, the Department of Justice, or the Office of the Inspector General of the HHS, as well as state authorities.

It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information. Moreover, with passage of the Pre-Approval Information Exchange Act, or PIE Act, in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance, but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products.

Expedited Review Programs

The FDA is authorized to expedite the review of applications in several ways. None of these expedited programs, however, changes the standards for approval but each may help expedite the development or approval process governing product candidates.

- *Fast Track Designation*. Candidate products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track application before the application is complete, a process known as rolling review.

- *Breakthrough therapy designation*. To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.

- *Priority review*. A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. FDA aims to complete its review of priority review applications within six months as opposed to ten months for standard review.

- *Accelerated approval*. Biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials. With passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to the FDA every six months until the study is completed; and use expedited procedures to withdraw accelerated approval of an NDA or BLA after the confirmatory trial fails to verify the product candidate's clinical benefit. Further, FDORA requires the FDA to publish on its website "the rationale for why a post-approval study is not appropriate or necessary" whenever it decides not to require such a study upon granting accelerated approval.

- *Regenerative advanced therapy*. With passage of the 21st Century Cures Act, or the Cures Act, in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

U.S. Orphan Drug Designation and Exclusivity

A gene therapy product may qualify for orphan drug designation, or ODD, under the Orphan Drug Act, if it is intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. ODD must be requested before submitting a BLA. After the FDA grants ODD, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. ODD entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. ODD does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a gene therapy product that has ODD receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same gene therapy product for the same indication for seven years, except in limited circumstances, such as not being able to supply the product for patients or showing clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of a different gene therapy for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan-drug exclusivity also could block the approval of one of our products for

seven years if a competitor obtains approval of the same gene therapy as defined by the FDA. In September 2021, the FDA issued final guidance describing its current thinking on when a gene therapy product is the "same" as another product for purposes of orphan exclusivity. Under the guidance, if either the transgene or vector differs between two gene therapy products in a manner that does not reflect "minor" differences, the two products would be considered different drugs for orphan drug exclusivity purposes. The FDA will determine whether two vectors from the same viral class are the same on a case-by-case basis and may consider additional key features in assessing the sameness.

In September 2021, the Court of Appeals for the 11th Circuit held that, for the purpose of determining the scope of market exclusivity, the term "same disease or condition" in the statute means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, the FDA announced that, in matters beyond the scope of that court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved.

Orphan drug products are also eligible for Rare Pediatric Disease Designation if greater than 50% of patients living with the disease are under age 18. A priority review voucher, or PRV, will be given to the sponsor of a product with a Rare Pediatric Disease Designation at the time of product approval that is transferable to another company. A PRV is a voucher that the FDA issues to a sponsor of a rare pediatric disease or tropical disease product application at the time of the marketing application approval. Vouchers are transferable to other sponsors that may apply it to their new drug applications or BLAs. A PRV entitles the holder to designate a single human drug application submitted under Section 505(b)(1) of the FDCA or Section 351 of the PHSA as qualifying for a priority review. An FDA priority review may expedite the review process of a marketing application reducing the review time from ten months after formal acceptance of the file to six months after formal acceptance of the file. Applying the PRV to a marketing application does not ensure the FDA's approval of the marketing application and all requirements supporting the safety and efficacy of the product must be met.

Biosimilars and Exclusivity

When a biological product is licensed for marketing by FDA with approval of a BLA, the product may be entitled to certain types of market and data exclusivity barring FDA from approving competing products for certain periods of time. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, was enacted in the United States and included the Biologics Price Competition and Innovation Act of 2009, or the BPCIA. The BPCIA amended the PHSA to create an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. To date, the FDA has approved a number of biosimilars and several interchangeable biosimilar products. In December 2022, Congress clarified through FDORA that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. Approval of a 351(k) application may not be made effective until twelve years after the date of first licensure of the reference product, which under the statute excludes the date of licensure of supplements and certain other applications. Additionally, a 351(k) application for a biosimilar or interchangeable biological product cannot be submitted for review until four years after the date on which the reference product was first licensed under section 351(a) of the PHSA. Even if a product is considered to be a reference product eligible for exclusivity, however, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well controlled clinical trials to demonstrate the safety, purity, and potency of their product. There have been recent government proposals to reduce the twelve-year reference product exclusivity period, but none has been enacted to date. At the same time, since passage of the BPCIA, many states have passed laws or amendments to laws, which address pharmacy practices involving biosimilar products.

U.S. Patent Term Restoration

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application, less any time the sponsor failed to act with due diligence. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

Other Healthcare Laws

Although we currently do not have any products on the market, we will be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states in which we conduct our business, if and when our product candidates are approved by the FDA and subject to federal healthcare reimbursement. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations. In addition, the U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of biologic products, limiting coverage and reimbursement for medical products and other changes to the healthcare system in the United States. In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for pharmaceutical products under government healthcare programs. The ACA is intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add transparency requirements for the healthcare and health insurance industries, impose taxes and fees on the health industry and impose additional health policy reforms. Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for products under government healthcare programs. Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which will remain in effect through 2031 pursuant to the Coronavirus Aid, Relief and Economic Security Act, or

CARES Act. These Medicare sequester reductions were reduced and suspended through the end of June 2022, with the full 2% cut resuming thereafter. The American Taxpayer Relief Act of 2012, which was enacted in January 2013, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers, and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a United States District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the TJCA, the remaining provisions of the ACA are invalid as well. The United States Supreme Court heard this case on November 10, 2020 and, on June 17, 2021, dismissed this action after finding that the plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Although the previous administration took executive actions to undermine or delay implementation of the ACA, those actions were rescinded with issuance of an Executive Order on January 28, 2021 by President Biden, which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

Pharmaceutical Price Reform

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent United States congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, the Centers for Medicare & Medicaid Services, or CMS, issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription products from Canada into the United States. The final rule is currently the subject of ongoing litigation, but at least six states (Vermont, Colorado, Florida, Maine, New Mexico, and New Hampshire) have passed laws allowing for the importation of products from Canada with the intent of developing SIPs for review and approval by the FDA. Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1,

2022, but with passage of the Inflation Reduction Act of 2022, or IRA, it has been delayed by Congress to January 1, 2032.

On July 9, 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. To address these costs, the executive order directs HHS to create a plan within 45 days to combat "excessive pricing of prescription drugs and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such drugs, and to address the recurrent problem of price gouging." Thereafter, on September 9, 2021, HHS released its plan to reduce drug prices. The key features of that plan are to: (a) make drug prices more affordable and equitable for all consumers and throughout the health care system by supporting drug price negotiations with manufacturers; (b) improve and promote competition throughout the prescription drug industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments.

More recently, on August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription product and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our

operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials, e.g., a clinical trial application for each clinical trial for each EU country in which the trial is conducted; a clinical trial notification is required in Japan.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Our Corporate Information

We were incorporated under the laws of Delaware in June 2013. Our principal executive offices are located at 64 Sidney Street, Cambridge, MA 02139. Other operations, including laboratory space, are located at 75 Hayden Avenue, Lexington, MA. We lease our office and laboratory space, which consist of approximately 26,148 square feet located in Cambridge, Massachusetts and 32,142 square feet located in Lexington, MA. Our lease in Cambridge expires in 2026 and our lease in Lexington expires in 2031.

Employees and Human Capital Resources

As of December 31, 2022, we employed 125 full-time employees in the United States, including 94 in research and development positions and 31 in general and administrative positions. Approximately 40 of our employees have either an MD or PhD degree. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective-bargaining arrangements. We consider our employee relations to be positive.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate our employees and directors and selected consultants through the granting of stock-based compensation awards.

Available Information

Our Internet address is http://www.voyagertherapeutics.com. We make available, free of charge, on or through our website our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy statements and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities and Exchange Act as soon as reasonably practicable after such material is electronically filed with or furnished to the Securities and Exchange Commission, or the SEC. The information on our website is not part of this Annual Report for the year ended December 31, 2022.

ITEM 1A. RISK FACTORS

The following risk factors and other information in this Annual Report on Form 10-K, including our financial statements and related notes thereto, should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see the discussion under the caption "Forward-Looking Statements" in this Annual Report on Form 10-K for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Capital

We have a history of incurring significant losses and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain consistent profitability.

We are an early-stage gene therapy company and have not yet generated revenues from the sales of our product candidates. All of our product candidates are in the early stages of development. Investment in biotechnology companies is highly speculative because it entails substantial upfront capital expenditures and significant risk that any product candidates will fail to be safe and efficacious, obtain regulatory approval or become commercially viable. We have not yet demonstrated the ability to complete any clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. We continue to incur significant expenses related to research and development, and other operations in order to commercialize our product candidates. We have a history of incurring significant operating losses. We had net losses of $46.4 million and $71.2 million for the year ended December 31, 2022 and December 31, 2021, respectively. As of December 31, 2022, we had an accumulated deficit of $393.5 million.

We historically have financed our operations primarily through private placements of our redeemable convertible preferred stock, public offerings of our common stock, and strategic collaborations, including our prior collaborations with Sanofi Genzyme Corporation, or Sanofi Genzyme, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company, and our ongoing collaborations with Neurocrine Biosciences, Inc., or Neurocrine; our option and license agreement, or the Pfizer Agreement, with Pfizer Inc., or Pfizer; and our option and license agreement, or the Novartis Agreement, with Novartis Pharma, AG, or Novartis. We refer to our ongoing collaborations with Neurocrine collectively as the Neurocrine Collaborations.

To date, we have devoted substantially all of our financial resources to building our gene therapy platform, selecting product programs, conducting research and development, including preclinical development of our product candidates, building our intellectual property portfolio, building our team, and establishing strategic collaborations. We expect that it could be several years before we have a commercialized product, if ever. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We also anticipate the cost of goods and services and the levels of compensation paid to employees will increase due to inflationary conditions existing in the general economy. The net losses we incur may fluctuate significantly from quarter to quarter.

We anticipate that our expenses will increase substantially if, and as, we:

- conduct preclinical development activities and initiate investigational new drug, or IND, application-enabling studies and clinical trials in connection with our tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques by continuing to develop our proprietary antibodies and vectorized antibody platform;
- increase our investment in and support for TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA), our proprietary discovery platform to facilitate the selection of AAV capsids

and expand our investment to discover TRACER capsids with broad tropism in central nervous system, or CNS and other tissues with cell-specific transduction properties for particular therapeutic applications;

- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs, including our FA Program pursuant to a collaboration with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement, and our GBA1 gene therapy program pursuant to our collaboration and license agreement with Neurocrine entered into on January 8, 2023, or the 2023 Neurocrine Collaboration Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;
- seek marketing and regulatory approvals for any of our product candidates that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- expand our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- increase our product liability and clinical trial insurance coverage as we expand our clinical trials and commercialization efforts; and
- continue to operate as a public company.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses. Our expenses will increase if:

- we are required by the FDA or the European Medicines Agency, or EMA, or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;
- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs; or
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

To become and remain profitable, we must develop and commercialize, alone or with our collaborators, product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities include completing preclinical studies and clinical trials of our product candidates; obtaining marketing approval for these product candidates; contracting with third parties with expertise in current good manufacturing practices, or cGMPs, to manufacture our product candidates at clinical and commercial scale; marketing and selling those products that are approved; satisfying any post-marketing requirements and achieving an adequate level of market acceptance of and obtaining and maintaining adequate coverage and reimbursement from third-party payors for such products; and protecting our rights to our intellectual property portfolio. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability.

If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause our stockholders to lose all or part of their investment.

We may not be able to generate sufficient revenue from the commercialization of our product candidates and may never be consistently profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with our collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future product candidates. All of our product candidates are in the early stages of development. We do not anticipate generating revenues from product sales for at least the next several years, and we may never succeed in doing so. Our ability to generate future revenues from product sales depends heavily on our and our collaborators' and licensors' success in:

- completing preclinical and clinical development of our product candidates or product candidates incorporating our licensed capsids or other technologies and identifying new product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we or they complete clinical trials;
- launching and commercializing product candidates for which we or they obtain regulatory and marketing approval by establishing a sales, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- obtaining and maintaining adequate coverage and reimbursement by government and third-party payors for our product candidates if and when approved;
- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that have the financial, operating and technical capabilities to provide adequate products and services, in both amount and quality, to support clinical development and the market demand for our or their product candidates, if and when approved;
- obtaining an adequate level of market acceptance of our or their product candidates as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, option, licensing, or other arrangements into which we may enter and performing our obligations in such collaborations;
- obtaining, maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party claims of interference or infringement; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could

increase beyond expectations if we are required by the FDA, EMA, or other regulatory authorities to redesign or modify preclinical studies or clinical trials or to perform preclinical studies or clinical trials in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.

We expect our expenses to increase over time in connection with our ongoing and planned activities, particularly as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We also continue to incur costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our research and development programs or any future commercialization efforts.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2022, our cash, cash equivalents, and marketable securities were $118.8 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2022, together with the upfront payment received in February 2023 in connection with the 2023 Neurocrine Collaboration Agreement along with amounts expected to be received as reimbursement for development costs under our collaboration and license agreements with Neurocrine, will enable us to meet our planned operating expenses and capital expenditure requirements into 2025.

Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and option and license agreements and any similar arrangement we may enter into in the future, including any research and development costs for which we are responsible, and our receipt of any future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty payments, under any collaboration or license agreements to which we might become a party, such as our license agreement with Touchlight IP Limited, or Touchlight, which we refer to as the Touchlight License Agreement;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;

- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company and maintaining adequate product, clinical trial, and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive, and uncertain process that takes years to complete. We may never generate the necessary data or results required to maintain the financial support of our collaborators or obtain marketing approval and achieve product sales. In the event we are unable to achieve milestones necessary to demonstrate progress on those programs, a current or future collaboration partner or licensor may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Our ability to develop a product candidate for any of our lead gene therapy or other biological therapy programs may take longer than we anticipate, or may not happen at all, and could require funding at a level higher than we expect. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration or option and license agreements will be derived from sales of products that may not be commercially available for many years, if at all. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing and business development to achieve our business objectives. Adequate additional financing or business development transactions may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate product revenues sufficient to achieve consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements. We do not have any committed external source of funds other than the amounts we are entitled to receive from our collaboration partner Neurocrine for the reimbursement of certain research and development expenses, the achievement of specified regulatory and commercial milestones, and royalty payments under the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement and the amounts we are entitled to receive from our licensors Pfizer and Novartis for the achievement of specified development, regulatory, and commercialization milestones and royalty payments under the applicable option and license agreements. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted. The amount of stockholder dilution will be affected by the size of each securities offering and the offering price for the securities sold. The offering price will likely reflect the prevailing market price for our securities, with dilution increasing as the prevailing market price for our securities decreases. The terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our common stock to decline. Further, our existing stockholders may not agree with the terms of such financings.

If we raise additional funds through collaborations, strategic alliances, or option and license arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds

through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves. Such collaborations, alliances, or option and license arrangements could therefore cause the market price of common stock to decline.

The preclinical stage of our development efforts may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

Our operating history to date has been limited to building our team, business planning, raising capital, establishing our intellectual property portfolio, determining which neurological diseases to pursue, advancing our product candidates including delivery and manufacturing and conducting preclinical studies and early-phase clinical trials. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had an operating history that included the late stage of clinical development, completion of clinical development, or commercialization of one or more product candidates. All of our active product candidates are currently in preclinical development.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors such as the regulatory setbacks that previously occurred in prior clinical programs we have run including the VY-AADC Program for Parkinson's disease and the VY-HTT01 Program for Huntington's disease, each of which was put on clinical hold by the FDA. These and other events that are part of our operating history may impact our ability to operate our business and to raise capital. All of our product candidates are in the early stages of development. To achieve our current goals, we will need to transition in the future from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, our stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

Risks Related to the Development and Regulatory Approval of Our Product Candidates

Our AAV gene therapy and other biological therapy product candidates are based on a proprietary technology and, in several disease areas, unvalidated treatment approaches, which makes it difficult and potentially infeasible to predict the duration and cost of development of, and subsequently obtaining regulatory approval for, our product candidates.

We have concentrated our research and development efforts to date on our gene therapy platform, identifying our initial targeted disease indications, and our initial product candidates. Our future success depends on our successful development of viable AAV gene therapy product candidates. Each of the product candidates we are advancing, either alone or together with our strategic collaborators, is currently in preclinical development.

AAV gene therapies are a relatively new technology. We cannot accurately predict when or if any of our product candidates will prove effective or safe in humans or whether these product candidates will receive marketing approval. Additionally, there can be no assurance that we will not experience problems or delays in the preclinical testing or development of our product candidates and that such problems or delays will not cause unanticipated costs, or that any such problems or delays can be solved in a timely or profitable basis, if at all. We also may experience unanticipated problems or delays in expanding our manufacturing capacity.

The clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the product candidate. The regulatory approval process for novel product candidates such as gene therapies can be more expensive and take longer than for other, better known or more extensively studied product candidates. Until August 2017, the FDA had never approved an AAV gene therapy product. Since that time, it has approved a limited number of gene therapy products including Hemgenix, an AAV gene therapy

product by CSL Behring LLC for adult patients with Hemophilia B (congenital Factor IX deficiency), Luxturna, an AAV gene therapy product by Spark Therapeutics, Inc. (acquired by F. Hoffmann-La Roche Ltd., or Roche, in 2019), or Spark, for patients with an inherited form of vision loss, and Zolgensma, an AAV gene therapy product by Avexis, a Novartis company, for pediatric patients with spinal muscular atrophy. In Europe, a similarly limited number of AAV gene therapy products including Hemgenix, Luxturna, and Zolgensma, as well as Upstaza by PTC, Roctavian by BioMarin Pharmaceuticals, Inc., and Glybera by uniQure N.V., or uniQure, have been granted marketing authorization; however, uniQure decided not to pursue renewal of such authorization in 2017 and has since withdrawn Glybera from the European market.

It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or how long it will take to commercialize our product candidates. The few regulatory approvals to date may not be indicative of what the FDA, EMA, or other regulatory authorities may require for approval or whether different or additional preclinical studies or clinical trials may be required to support regulatory approval in a particular jurisdiction. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects may be harmed.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to modify current studies or perform additional studies or increase our development costs, which in turn may force us to delay, limit, or terminate certain of our programs.

The Center for Biologics Evaluation and Research, or CBER, of the FDA regulates biological products for human use. The Office of Tissues and Advanced Therapies, or OTAT, formerly known as the Office of Cellular, Tissue and Gene Therapies, within CBER reviews gene therapy and related products and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review.

NIH-funded institutions need to have their institutional biosafety committee, or IBC, as well as their institutional review board, or IRB, review proposed clinical trials to assess the safety of the trial. If the protocol for such a trial was amended, it would need to be re-reviewed by the respective institutional IRBs of each institution. Any delay in or failure to obtain institutional IRB approval for any protocol or protocol amendment could delay, interrupt, or limit the conduct of the clinical trial at one or more participating clinical trial sites.

Adverse or unforeseen developments in clinical trials of gene therapy products conducted by us or others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, EMA and local health authorities of individual countries within the European Union may issue new guidelines concerning the clinical development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. The EMA and agencies at both the federal and state level in the United States have expressed an interest in further regulating new biotechnologies, including gene therapy. In addition, gene therapy products are considered genetically-modified organism, or GMO, products and are regulated as such in each country. Designation of the type of GMO product and subsequent handling and disposal requirements can vary across countries and is variable throughout the European Union. Addressing each specific country requirement and obtaining approval to commence a clinical trial in these countries could result in delays in starting, conducting, or completing a clinical trial. Similar issues could be faced in other regions of the world including the Asia-Pacific region.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. For example, we requested feedback from the FDA on, among other matters, the regulatory pathway for VY-AADC (NBIb-1817) and the design of the proposed pivotal program. We had multiple interactions with the FDA and received feedback from the FDA that, in a disease such as Parkinson's, two adequate and well-controlled clinical trials is suggested.

Any inability to receive timely, actionable feedback from regulatory authorities could also delay or otherwise hinder our development efforts. In October 2020, the FDA notified us that the IND application for our planned Phase 1 and 2 clinical trial to evaluate VY-HTT01 in patients with Huntington's disease was placed on clinical hold pending the resolution of certain information requests regarding chemistry, manufacturing, and controls, or CMC, matters. We had previously sought and received FDA feedback on the VY-HTT01 development program in a pre-IND meeting in 2017. Because the FDA only grants one pre-IND meeting per product in a given indication, however, we were unable to have additional formal consultations with the FDA prior to our submission of our IND application in September 2020 concerning changes to the program since our 2017 meeting. Although we decided in August 2021 not to commence the VYTAL Phase 1 and 2 clinical trial for VY-HTT01 once we had resolved the clinical hold, these and other regulatory delays may require us to incur additional clinical development costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue from our product candidates.

We plan to continue to seek and incorporate FDA guidance in our ongoing development plans for each of our potential clinical candidates. If we fail to consult or solicit guidance from regulators or are unable to obtain sufficiently frequent or detailed guidance from regulators, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of increased or lengthier regulatory approval process and further restrictions on development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Results from preclinical studies and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials.

All of our product candidates are in early stages of development, and the risk of failure is high. Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Our product candidates may fail to show the desired safety and efficacy in preclinical testing or clinical development despite demonstrating promising results in earlier preclinical studies or clinical trials. In addition, the outcome of preclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials. For example, despite data we believed was promising from the earlier PD-1101 Phase 1b clinical trial and from the separate PD-1102 Phase 1 clinical trial evaluating the delivery of VY-AADC (NBIb-1817), we and our strategic collaborator Neurocrine did not receive favorable data, and were ultimately unable to complete, the RESTORE-1 Phase 2 clinical trial evaluating VY-AADC (NBIb-1817) for the treatment of Parkinson's disease. Similarly, interim results generated from clinical trials do not necessarily predict final results, and results from one completed clinical trial may not be replicated in a subsequent clinical trial with a similar study design. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be sustained or repeated in larger and more advanced clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

There is a high failure rate for product candidates proceeding through preclinical studies and clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results, if these results are not reproducible, or if our products show diminishing activity over time, our products may not receive approval from the EMA or the FDA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late-stage clinical trials with larger patient populations could harm our business and we may never succeed in commercialization or generating product revenue.

We may in the future conduct clinical trials for product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations.

To date, we have only conducted clinical trials in the United States. However, we may in the future choose to conduct one or more of our clinical trials or include sites in current or future clinical trials outside the United States.

Although the FDA may accept data from sites or clinical trials outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, the clinical trial must be well-designed and conducted and performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials or trial sites are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials or trial sites also complied with all applicable U.S. laws and regulations. If the FDA does not accept the data from any trial or trial site outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and would delay or permanently halt our development of the applicable product candidates.

Other risks inherent in conducting international clinical trials or using international trial sites include:

- foreign regulatory requirements that could restrict or limit our ability to conduct our clinical trials;
- the administrative burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment;
- the failure of enrolled patients to adhere to clinical protocols or inadequate collection and assessment of clinical data as a result of differences in healthcare services or cultural customs;
- foreign exchange fluctuations;
- diminished or loss of protection of intellectual property in the relevant jurisdiction; and
- political, economic, environmental, and health risks relevant to specific foreign countries, including risks related to natural disasters or disease outbreaks.

We are early in our development efforts. All of our active product candidates are currently in preclinical development. We may encounter substantial delays or difficulties in commencement, enrollment or completion of our preclinical studies or clinical trials, or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.

We are early in our development efforts, and all of our active product candidates are currently in preclinical development. Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. To conduct clinical trials, we must first complete preclinical testing and studies to support IND applications or similar applications in other jurisdictions. We cannot be certain of the timely completion or successful outcome of our preclinical testing and studies. Our ability to complete our preclinical testing and studies is contingent on, among other things, our ability to source animals and other supplies required for the conduct of such testing and studies. If we are unable to obtain such supplies, we may be unable to complete such preclinical testing and studies in a timely manner or at all. For example, some of our IND-enabling toxicology and other studies require certain non-human primates that are customarily imported from the People's Republic of China, or the PRC, and Cambodia. The supply of these non-human primates is currently constrained due to factors such as their limited worldwide availability, trade relations between the United States and the PRC, and heightened scrutiny of non-human primates originating from Cambodia following allegations in late 2022 that certain Cambodian businesses and government officials may have engaged in the smuggling of non-human primates. We have encountered, and may continue to encounter, delays in obtaining a sufficient supply of

such non-human primates to enable the conduct of our preclinical studies and testing. In addition, we may need to conduct preclinical studies utilizing non-human primates located in testing facilities outside of the United States. Utilizing such facilities will require us to observe export control regulations for the shipment of vectors and transgenes and import controls for the shipment of samples to us for evaluation and storage, which controls we may not be to satisfy, or may result in delay or additional expense. Our inability to obtain access to a sufficient supply of these non-human primates in a timely manner or at all may impair our ability to complete preclinical testing and studies to support IND applications or similar applications in other jurisdictions or delay the submission of such applications.

Additionally, we cannot predict if the FDA or similar regulatory authorities outside the United States will accept our planned clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our preclinical and clinical programs. In connection with our VY-HTT01 Program for the treatment of Huntington's disease, for example, we were unable to predict what the FDA would require and were unable to obtain a second pre-IND meeting with the FDA to discuss the product candidate's regulatory pathway with the FDA. As a result, in October 2020, the FDA notified us that the IND application for the planned Phase 1 and Phase 2 clinical trial to evaluate VY-HTT01 had been put on clinical hold.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. For example, in December 2022, with the passage of Food and Drug Omnibus Reform Act, known was FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, actions plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In addition to these requirements, the legislation directs the FDA to issue new guidance on diversity action plans. Similarly, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the Clinical Trials Directive required a separate clinical trial application, or CTA, to be submitted in each member state, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application to all member states concerned. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial failure can occur at any stage of testing. Similarly, there may be delays or difficulties in our initiation of future clinical trials. Due to the additional regulatory uncertainties associated with gene therapy products, for example, we did not initiate the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817) as a treatment for Parkinson's disease until we met with OTAT to discuss our proposed trial design and overall development plan. While we received OTAT's feedback and incorporated it as appropriate in our plans, the clinical trial as designed may not achieve the prospectively defined primary clinical endpoints or provide a favorable benefit to risk ratio to support a BLA, filing or approval.

We also have very limited historical experience with clinical trials. Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner or at all pursuant to the requirements of the FDA, EMA, or other regulatory authorities. Patient enrollment and trial completion are affected by many factors including:

- perceived risks and benefits of AAV gene therapy approaches for the treatment of neurological and other diseases;

- formulation changes to our product candidates, which may require us to conduct additional clinical studies to bridge our modified product candidates to earlier versions;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- patients with preexisting antibodies to the gene therapy vector that preclude their participation in the trial;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- lack of adequate compensation of patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- our ability to locate appropriately trained physicians to conduct such clinical trials, particularly for clinical trials requiring lengthy and highly complex surgical protocols, the performance of which may only be possible at major academic medical centers or specialized surgical centers;
- willingness of patients to participate in a placebo-controlled trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Further, we plan to seek marketing approvals in the United States, the European Union and other jurisdictions, which may require that we conduct clinical trials in foreign countries. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with clinical research organizations, or CROs, and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; and

- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials in some or all localities, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities or collaborators on trial design, implementation, management, or other aspects of the clinical trial;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;
- as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites or the decision by us or our collaborators, or the requirement of regulators or IRBs to suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- failure by us, our collaboration partners, any CROs we engage, or any other third parties to adhere to clinical trial protocols or regulatory requirements;
- failure by us, our collaboration partners, any CROs we engage, or any other third parties to perform in accordance with the FDA's good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- insufficient or inadequate supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial at a rate higher than we anticipate;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- receipt of negative or inconclusive clinical trial results;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;

- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- the cost of clinical trials of our product candidates may be greater than we anticipate.

Any inability to successfully initiate or complete preclinical studies and clinical trials could result in additional costs and potential delays to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. For example, our decision to refocus our Huntington's disease program means we must conduct new preclinical studies, prepare a new IND, submit it to the FDA, and resolve any potential FDA objections before enrolling our first patient in a new clinical trial. In addition, if we make manufacturing or formulation changes to our product candidates, such as our previous transition from an HEK 293-based production system to a baculovirus/Sf9 AAV production system or as a result of unanticipated clinical trial results, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or SAEs associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if we are able to do so at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a Risk Evaluation and Mitigation Strategy, or REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued or otherwise become party to dispute proceedings; or
- experience damage to our reputation.

Our product candidates or the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

In past clinical trials that were conducted by others using non-AAV gene therapy vectors, several significant side effects were caused by gene therapy treatments, including reported cases of leukemia and death. Other potential side effects could include an immunologic reaction and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If our vectors demonstrate a similar adverse effect, or other adverse effects, we may be required to halt or delay further clinical development of our product candidates or withdraw the product from the market post-approval. For example, in a recently published review of patients with

hepatocellular carcinomas, it was shown that a small subset contained an integrated genome sequence of wild-type AAV2 and it was suggested that AAV2 may be associated with insertional oncogenesis.

In addition to side effects caused by the product candidate, the administration process or related procedures also could cause side effects. If in the future we are unable to demonstrate that such side effects were caused by the administration process or related procedures or are unable to modify the trial protocol adequately to address such side effects, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. For products that "knock down" or reduce the expression of a gene or the production of its encoded protein, their effects on other parts of the body, or "off target" effects, could result in unforeseen toxicity. Even if we are able to demonstrate that any future SAEs are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits outweigh its risks. We believe that the likelihood of the FDA requiring a REMS may be higher for treatments with more invasive routes of administration such as direct delivery through brain surgery. Such REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners or the limitation of the use of the product to specifically trained neurosurgeons and/or certain centers. Furthermore, adverse events which were initially considered unrelated to the study treatment of the clinical trial may later be found to be caused by the study treatment. If we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and could significantly harm our business, prospects, financial condition and results of operations.

We may be unable to obtain orphan drug designation or exclusivity for any of our product candidates for which we seek such designation. If our competitors are able to obtain orphan drug exclusivity for products that constitute the "same drug" and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. For products for which we may obtain orphan drug designation or exclusivity, we may be unable to prevent the approval or marketing authorization of other similar products based upon regulatory decisions regarding product "sameness".

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, or the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the

cost of developing the drug or biological product will be recovered from sales in the United States. In the European Union, EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we may be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the new drug application or BLA sponsor submits pediatric data that adequately respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to nine years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

We believe that certain of our current programs may qualify for orphan drug designation. Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs or biological products can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug or biological product for the same condition if the FDA concludes that the other drug or biological product is not the "same drug" or biological product or even if it is, the FDA determines that it is clinically superior in that it is shown to be safer or more effective or makes a major contribution to patient care. In September 2021, the FDA issued final guidance describing its current thinking on when a gene therapy product is the "same" as another product for purposes of orphan exclusivity. Under the guidance, if either the transgene or vector differs between two gene therapy products in a manner that does not reflect "minor" differences, the two products would be considered different drugs for orphan drug exclusivity purposes. The FDA will determine whether two vectors from the same viral class are the same on a case-by-case basis and may consider additional key features in assessing the sameness.

In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority.

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies, particularly in light of a decision from the U.S. Court of Appeals for the Eleventh Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the Court of Appeals concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have sought and may in the future seek a breakthrough therapy designation for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the drugs or biological products no longer meet the conditions for qualification.

A potential regenerative medicine advanced therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have sought and may in the future seek a regenerative medicine advanced therapy, or RMAT, designation for some of our product candidates. Under the 21st Century Cures Act, or the Cures Act, to be eligible to receive RMAT designation from the FDA, a product candidate must be (a) considered a "regenerative medicine therapy" as defined in the Cures Act; (b) intended to treat, modify, reverse, or cure one or more serious or life-threatening diseases or conditions; and (c) indicated, in preliminary clinical evidence, to have the potential to address unmet medical needs for such diseases or conditions. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition in the Cures Act of a regenerative medicine therapy.

The RMAT program is intended to facilitate efficient development and expedite review of such therapies. A new drug application or a BLA for a product candidate that has received an RMAT designation may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A product candidate that has received an RMAT designation that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

RMAT designation is within the discretion of the FDA. Accordingly, even if we believe one of our other product candidates meets the criteria for RMAT designation, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of RMAT designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that a product candidate that received RMAT designation no longer meets the conditions for designation. Alternatively, we or our collaborative partners may decide not to proceed with the clinical development of a product candidate that has previously received RMAT designation or decide to pursue such product candidate for an indication for which it has not received RMAT designation.

Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of our product candidate.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical need for this condition, the drug sponsor may apply for FDA fast track designation. We have sought and may in the future seek such a designation for our product candidates. A fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. Thus, fast track products may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from a product candidate's clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of our product candidate.

If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the FDA's goal to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we or any current or future collaborators are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we or they may not be able to commercialize our products, and our ability to generate revenue may be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, export and import, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA and comparable regulatory authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process.

Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product

candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive; may take many years if additional clinical trials are required, if approval is obtained at all and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. In the United States, for example, the application user fee to obtain FDA review of a marketing application is more than $3.1 million, and may be higher in the future. Changes in marketing approval policies during the development period, in or the enactment of additional statutes or regulations, or in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any current or future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Accordingly, if we or any current or future collaborators experience delays in obtaining approval or if we or they fail to obtain or retain approval of our product candidates and devices, the commercial prospects for our product candidates may be harmed and our ability to generate revenues could be materially impaired.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storing, advertising, promoting, sampling, record-keeping and submitting safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or such regulatory authority disagrees with the promotion, marketing or labeling of that product, the regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;

- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application, or any supplements thereto, submitted by us or our collaboration partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and adversely affect our business, financial condition, results of operations and prospects.

In addition, FDA policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would harm our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition and our ability to successfully market or commercialize our product candidates.

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop into products and commercialize may compete with existing therapies and new therapies that may become available in the future. While we believe that our gene therapy platform, vectorized antibody platform, product programs, product candidates and scientific expertise in the fields of gene therapy and neuroscience provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

We are aware of several companies focused on developing AAV gene therapies in various indications, including AavantiBio, Inc. (acquired by Solid Biosciences, Inc., or Solid), Abeona Therapeutics, Inc., Adverum Biotechnologies, Inc., Aevitas Therapeutics, Inc., Akouos, Inc. (acquired by Eli Lilly and Company, or Eli Lilly), Alcyone Therapeutics, Inc., Amicus Therapeutics, Inc., Apic Bio, Inc., Applied Genetic Technologies Corporation (acquired by Syncona Limited), Asklepios BioPharmaceutical, Inc., or AskBio (acquired by Bayer), Audentes Therapeutics, Inc. (acquired by Astellas Pharma Inc.), Biogen, Inc., or Biogen, Brain Neurotherapy Bio, Inc. (merged with AskBio), BioMarin, Encoded Therapeutics, Inc., GenSight Biologics SA, Homology Medicines, Inc., LEXEO Therapeutics, Inc., LogicBio Therapeutics, Inc. (acquisition by AstraZeneca announced), Lysogene SA, MeiraGTx Ltd., or MeiraGTx, Neurogene, Inc., Novartis Gene Therapies, Inc. (formerly AveXis, Inc.), Passage Bio, Inc., Pfizer, Prevail Therapeutics Inc. (acquired by Eli Lilly), PTC, REGENXBio Inc., Sarepta Therapeutics, Inc., Solid, Spark, StrideBio, Inc., Taysha Gene Therapies, Inc. and uniQure, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

We expect that our TRACER discovery platform and preclinical programs will compete with a variety of therapies in development, including:

- Our TRACER discovery platform will potentially compete with a variety of companies developing AAV capsids, including: 4D Molecular Therapeutics, Inc., Affinia Therapeutics Inc., Apertura Gene Therapy, LLC, Capsida Biotherapeutics, Inc., Capsigen Inc., Dyno Therapeutics, Inc., Kate Therapeutics, Inc., Shape Therapeutics Inc., and StrideBio, Inc.;

- Our program for diseases linked to GBA1 mutations will potentially compete with AAV gene therapies being developed by Prevail Therapeutics Inc. (acquired by Eli Lilly), Freeline Therapeutics Holdings plc, Pfizer, Biogen, Lysogene SA, and Coave Therapeutics SA;

- Our program for tauopathies including Alzheimer's disease, progressive supranuclear palsy, and frontotemporal dementia will potentially compete with tau antibodies being developed by Roche Genentech Inc. in collaboration with AC Immune SA, Eli Lilly, AbbVie, Biogen, Eisai Co., Ltd., Janssen Pharmaceuticals, Inc., UCB S.A., and several other companies, as well as an antisense oligonucleotide program being developed by Ionis in collaboration with Biogen;

- Our program for a monogenic form of ALS will potentially compete with Tofersen being developed by Biogen, in collaboration with Ionis, and gene therapies being developed by Novartis Gene Therapies, Inc. and Apic Bio, Inc.;

- Our treatment of Freidrich's ataxia under the FA Program will potentially compete with AAV gene therapies being developed by LEXEO Therapeutics, Inc., AavantiBio, Inc. (acquired by Solid), PTC, StrideBio, Inc. in collaboration with Takeda Pharmaceutical Company Limited, Pfizer, and Novartis Gene Therapies, Inc.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries, including recent transactions involving a number of gene therapy companies, may result in even more resources being concentrated among a smaller number of competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative agreements with large and established companies. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than us or may obtain orphan drug or other marketing exclusivity, which could result in our competitors establishing a strong market position before we are able to enter the market or reducing the number of available subjects for enrollment in our clinical trials to support regulatory submissions and approvals of our product. Additionally, technologies developed or acquired by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors. These third parties also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites, and registering patients for clinical trials.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. If we are not able to compete effectively against potential competitors, our business will not grow, and our financial condition and operations will be harmed.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials or manufacturing control requirements. In many countries outside the United States, a product candidate must be separately approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates in the European Union but obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Effective January 1, 2021, the United Kingdom is no longer part of the European Single Market and European Union Customs Union. A cooperation agreement was signed between the United Kingdom and the European Union in December 2020, which was applied provisionally beginning on January 1, 2021 and entered into force on May 1, 2021. The agreement addresses trade, economic arrangements, law enforcement, judicial cooperation and a governance framework including procedures for dispute resolution, among other things. As both parties continue to work on the rules for implementation, significant political and economic uncertainty remains about how the precise terms of the relationship between the parties will differ from the terms before withdrawal.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, the consequences of Brexit and the impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom remain unclear. As of January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or the MHRA, became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law, whereas Northern Ireland will continue to be subject to European Union rules under the Northern Ireland Protocol. The MHRA will rely on the Human Medicines Regulations 2012 (SI 2012/1916) (as amended), or the HMR, as the basis for regulating medicines. The HMR has incorporated into the domestic law of the body of European Union law instruments governing medicinal products that pre-existed prior to the United Kingdom's withdrawal from the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for our product candidates, which could significantly and materially harm our business.

We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

If approved, our product candidates that are licensed and regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the ACA, to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product In addition, the licensure of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still develop and receive approval of a competing biologic, so long as its BLA does not reply on the reference product, sponsor's data or submit the application as a biosimilar application.

We believe that any of the product candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our product candidates would have a material adverse impact on our business due to increased competition and pricing pressure.

Risks Related to Third Parties

To date, all of our revenue has been derived from our ongoing collaborations with Neurocrine, from our ongoing option and license arrangements with Pfizer and Novartis, and from our prior collaborations with Sanofi Genzyme, AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company. If any ongoing or future collaboration or option and license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed.

To date, all of our revenue has been derived from our ongoing collaborations with Neurocrine, our ongoing option and license arrangements with Pfizer and Novartis, and from our prior collaborations with Sanofi Genzyme and AbbVie. If any ongoing or future collaboration or option and license agreements were to be terminated, our business financial condition, results of operations and prospects could be harmed. For example, certain of our prior collaborations were terminated. As a result of the terminations of our collaborations with Sanofi Genzyme and AbbVie, we ceased to be eligible to receive option and milestone payments pursuant to the collaborations or to receive royalties in connection with any potential products developed under the collaborations.

On February 2, 2021, Neurocrine notified us that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program. This termination became effective August 2, 2021, which we refer to as the Neurocrine VY-AADC Program Termination Effective Date. The 2019 Neurocrine Collaboration Agreement remains in full force and effect for each other program thereunder. Upon the termination of the VY-AADC Program, the license granted by us to Neurocrine regarding the VY-AADC Program expired, and we regained worldwide

intellectual property rights to the VY-AADC Program in accordance with the collaboration agreement, and the restrictions on us to develop, manufacture or commercialize a gene therapy product directed to the targets specified in the VY-AADC Program terminated. If Neurocrine were to terminate the remainder of the 2019 Neurocrine Collaboration Agreement, we would become responsible for all research and development expenses relating to the remaining Neurocrine Programs and would not receive any future milestone payments or royalty payments under the 2019 Neurocrine Collaboration Agreement with respect to such programs.

On October 1, 2021, we entered into the Pfizer Agreement, pursuant to which we granted Pfizer options to receive an exclusive license to TRACER capsids to develop and commercialize certain AAV gene therapy candidates comprised of a TRACER capsid and specified transgenes to help treat respective central nervous system and cardiovascular diseases. Effective as of September 30, 2022, Pfizer exercised its option with respect to a capsid in connection with a gene therapy program for the potential treatment of an undisclosed rare neurologic, rare neurological disease, or the Pfizer Option Exercise. Under the terms of the Pfizer Agreement, pursuant to the Pfizer Option exercise, we are eligible to receive specified development, regulatory, and commercialization milestone payments following of up to an aggregate of $115.0 million for the first licensed product to achieve such milestones; specified sales milestone payments of up to an aggregate of $175.0 million per licensed product; and tiered, escalating royalties in the mid- to high-single digit percentages of annual net sales of each licensed product. Pfizer did not exercise its option to license a capsid for a specified cardiovascular disease target under the Pfizer Agreement. As a result, all rights to capsids for that cardiovascular disease target under the Pfizer Agreement have expired and have reverted to us, and we are not eligible to receive any potential future development, regulatory, commercialization, or sales milestone payments or potential royalties pursuant to the Pfizer Agreement in connection with such target.

In March 2022, we entered into the Novartis Agreement, pursuant to which we granted Novartis options to receive an exclusive license to TRACER capsids to develop and commercialize certain AAV gene therapy candidates comprised of a TRACER capsid and specified genetic payloads for specific genetic targets. Under the terms of the Novartis Agreement, we received an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its options to license novel capsids generated from our TRACER capsid discovery platform for use in gene therapy programs against two undisclosed neurologic disease targets. With Novartis' option exercise on two targets, we are entitled to receive a $25.0 million option exercise payment during the first half of 2023, and we are eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of Novartis products incorporating the licensed capsids. In addition, over the next 18 months, Novartis retains the right to expand the agreement to include options to license capsids for up to two additional rare CNS targets, subject to their availability, for a fee of $18.0 million per target. Under such an expansion, we would be eligible to receive a $12.5 million license option exercise fee for each target exercised, as well as future potential milestone payments per target and mid- to high-single-digit tiered royalties on products incorporating the licensed capsids. Novartis elected not to license a capsid for one CNS target under the Novartis Agreement prior to the expiration of the applicable option period. As a result we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this target, and all capsid rights with respect to that target have returned to us.

Our current collaborators or any future collaborator might not be successful in obtaining approvals for the product candidates arising from our collaboration or commercializing or manufacturing the resulting products. Further, such collaborator's objectives in connection with the collaboration may not be consistent with our best interests. With respect to the rights granted to a collaborator by us, the collaborator could take actions that may be adverse to us, or it could halt, slow, or deprioritize its development and commercialization efforts under the collaboration. In any such instances, our business, financial condition, results of operations and prospects could be materially harmed.

We may seek to enter into collaborations, and out-licensing transactions in the future with other third parties. If we are unable to enter into such collaborations or out-licensing transactions, or if these collaborations or out-licensing transactions are not successful, our business could be adversely affected.

We may seek to enter into additional collaborations in the future, including sales, marketing, distribution, development, option, licensing, and/or broader collaboration agreements. For example, we entered into the 2023 Neurocrine Collaboration Agreement for the development, manufacture and commercialization of the 2023 Neurocrine

Programs. Our likely collaborators, optionees, and licensees include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, biotechnology companies, and medical device manufacturers. However, we may not be able to enter into additional collaborations or option and license transactions on favorable terms or at all. Our ability to generate revenues from our collaborations and option and license transactions will depend on our and our collaborators', optionees', and licensees' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our collaborators, optionees, and licensees might have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our collaborator, optionee, or licensee is responsible could be harmful to the public perception and prospects of our gene therapy and vectorized antibody platforms.

Our relationship with any current or future collaborators, optionees, or licensees may pose several risks, including the following:

- collaborators, optionees, and licensees have significant discretion in determining the amount and timing of the efforts and resources that they will apply to these collaborations and option and license transactions;
- collaborators, optionees, or licensees may not perform their obligations as expected or desired;
- the preclinical studies and clinical trials conducted as part of these collaborations or by our licensees may not be successful;
- collaborators, optionees, or licensees may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on preclinical study or clinical trial results, changes in the collaborators', optionees', or licensees' strategic focus or available funding or external factors, such as an acquisition, which divert resources or create competing priorities;
- collaborators, optionees, or licensees may delay preclinical studies and clinical trials, provide insufficient funding for preclinical studies and clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new preclinical studies or clinical trials or require a new formulation of a product candidate for preclinical studies or clinical trials;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration or by a licensee and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- collaborators, optionees, or licensees could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators, optionees, or licensees believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates developed in collaboration with us or by a licensee may be viewed by our collaborators or licensees as competitive with their own product candidates or products, which may cause collaborators or licensees to cease to devote resources to the commercialization of our product candidates;
- a collaborator or licensee with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- disagreements with collaborators, optionees, or licensees, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to

additional responsibilities or expenses for us with respect to such product candidates (in the case of collaborations) or may result in litigation or arbitration, any of which would be time-consuming and expensive;

- collaborators, optionees, or licensees may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- disputes may arise with respect to the ownership or inventorship of intellectual property developed pursuant to our collaborations or option and license transactions;

- collaborators, optionees, or licensees may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

- the terms of our collaboration or license agreement may restrict us from entering into certain relationships with other third parties, thereby limiting our options; and

- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration and license agreements may not lead to the development or commercialization of product candidates in the most efficient manner, or at all. If our collaborations or option and license transactions do not result in the successful development and commercialization of products, or if one of our collaborators, optionees, or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration or option and license transactions. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed, and we may need additional resources to develop our product candidates. In the event we are unable to achieve milestones necessary to demonstrate progress on our programs relevant to our ongoing collaborations with Neurocrine, Neurocrine may be unwilling to fund these programs at the desired levels or at all, which could require us to fund these programs to a greater extent than we have expected, to decline to pursue certain program objectives or to discontinue one or more of the programs. Additionally, subject to its contractual obligations to us, if a collaborator, optionee, or licensee of ours were to be involved in a business combination, it might deemphasize or terminate the development or commercialization of any product candidate optioned or licensed to it by us. If one of our collaborators, optionees, or licensees terminates its agreement with us, we may find it more difficult to attract new collaborators, optionees, or licensees, and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this periodic report also apply to the activities of our collaborators, optionees, and licensees.

We will face significant competition in seeking appropriate collaborators, optionees, and licensees, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration or license agreement with any future collaborators, optionees, and licensees will depend, among other things, upon our assessment of the collaborator's, optionee's, or licensee's resources and expertise, the terms and conditions of the proposed collaboration or option and license transactions and the proposed collaborator's, optionee's, or licensee's evaluation of several factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator, optionee, or licensee may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration or option and license transaction could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators, optionees, or licensees. In addition, there have been a

significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators, optionees, and licensees.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations or option and license transactions and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our gene therapy and vectorized antibody platforms. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

We and our collaborators have relied, and we and our collaborators expect to continue to rely, on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if these third parties perform in an unsatisfactory manner, our business could be harmed.

We and our collaborators expect to rely on CROs, clinical trial sites, and other vendors to ensure our preclinical studies and clinical trials are conducted properly and on time. We and our collaborators may also engage third parties such as clinical data management organizations, medical institutions and clinical investigators to conduct or assist in our clinical trials or other preclinical and clinical research and development work. While we and our collaborators will have agreements governing their activities, we and our collaborators will have limited influence over their actual performance. We and our collaborators will control only certain aspects of our third-party service providers' activities. Nevertheless, we and our collaborators will be responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal, quality, regulatory and scientific standards. Our reliance on these third parties does not relieve us of our regulatory responsibilities. For example, the PD-1101 Phase 1b clinical trial of VY-AADC (NBIb-1817) and the separate PD-1102 Phase 1 clinical trial exploring the delivery of VY-AADC (NBIb-1817) using a posterior trajectory were conducted at several locations. Additionally, we had expected to initiate the planned VYTAL Phase 1 and 2 clinical trial for VY-HTT01 at multiple sites in the United States before our decision to refocus the Huntington's disease program. If any locations terminate a particular clinical trial, we or our collaborators would be required to find other parties or locations to conduct such clinical trial. We and our collaborators may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials. If we or our collaborators elect to internalize some or all activities related to the conduct of our preclinical studies or clinical trials that are currently performed by our third-party service providers, or if we or our collaborators are required to do so due to a service provider's termination of our relationship, then we or our collaborators may be required to source additional technology and personnel in order to perform the relevant activities. We and our collaborators may be unsuccessful in our efforts to internalize some or all relevant activities, either on the desired timeline or at all.

We, our collaborators, and our third-party service providers are required to comply with the FDA's good laboratory practices, or GLPs, and GCPs for conducting, recording and reporting the results of IND-enabling preclinical studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. We and our collaborators are also required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. The FDA enforces these GLPs and GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites, and laboratories at which the FDA may determine that our preclinical studies and clinical trials did not comply with GLPs or GCPs. If we, our collaborators, or our third-party service providers fail to comply with applicable GLPs or GCPs, the preclinical or clinical data generated in our future preclinical studies or clinical trials may be deemed unreliable and the FDA may require us to perform additional preclinical studies or clinical trials before approving the relevant INDs or marketing applications. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if we, our collaborators, or our third-party service providers fail to comply with these

regulations or fail to recruit a sufficient number of patients, we may be required to repeat such preclinical studies or clinical trials, which would delay the regulatory approval process. Failure to comply can also result in fines, adverse publicity, and civil and criminal sanctions.

Our third-party service providers are not our employees, and we and our collaborators are therefore unable to directly monitor whether or not they devote sufficient time, attention, expertise and resources to our clinical and nonclinical programs. These third-party service providers may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our third-party service providers do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our preclinical studies or clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

Risks Related to Manufacturing

Our gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies. To meet the requirements of our current and planned future trials we have developed a flexible manufacturing platform that is based on proprietary technology and provides a scalable process for preclinical and clinical AAV production. We are using HEK 293 cell manufacturing to support our preclinical research activities. We also have expertise with the baculovirus/Sf9 AAV production system, a technology for producing AAV gene therapy vectors at scale in insect-derived cells, which we have used for our clinical development activities in the past and may use in the future for clinical development activities. Both the HEK 293 cell manufacturing process and the baculovirus/Sf9 manufacturing process have been successfully transferred to our contract manufacturing organizations. The baculovirus/Sf9 manufacturing process has also been used by our contract manufacturing organizations in manufacturing clinical materials in accordance with the FDA's cGMPs. If we transition from the use of HEK 293 cell manufacturing for preclinical research activities to the use of the baculovirus/Sf9 AAV production system for clinical development activities, we could encounter transition-related difficulties such as the need to make manufacturing process adjustments, the need to change third-party contract manufacturers, issues with drug potency consistency, and adverse clinical reactions, which could lead us to incur additional costs or delays.

We presently contract with third parties for the manufacturing of our program materials. We have also built an onsite, state-of-the-art process research and development facility to enable the manufacturing of clinical quality AAV gene therapy vectors at research scale. We are currently assessing our manufacturing capabilities and, although we do not currently have our own clinical or commercial scale manufacturing, we may choose to build those capabilities. The use of contracted manufacturing and reliance on collaboration partners is relatively cost-efficient and eliminates the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements for our program materials. We expect third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. To meet our projected needs for commercial manufacturing, third parties with whom we currently work might need to increase their scale of production or we will need to secure alternate suppliers. We believe that there are alternate sources of supply for our program materials that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

To date, our third-party manufacturers have met our quality standards for our program materials. The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical study. If we or our manufacturers were to fail to comply with the FDA, EMA, or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Several of these raw materials, cells, and reagents are provided by a limited number of suppliers. Even though we aim to have backup supplies and suppliers of raw materials, cells, and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects on our manufacturing processes, including delays.

Delays in obtaining regulatory approval of our or our collaborators' manufacturing processes and facilities or disruptions in such manufacturing processes may delay or disrupt our commercialization efforts. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product.

Before we can begin to commercially manufacture a product candidate in our own facility, or the facility of a collaborator, we must obtain regulatory approval from the FDA for our manufacturing process and our collaborator's facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities. Until recently, no cGMP gene therapy manufacturing facility in the United States had received approval from the FDA for the manufacture of an approved gene therapy product and, therefore, the timeframe required for us to obtain such approval is uncertain. In addition, we must pass a pre-approval inspection of our or our collaborator's manufacturing facility by the FDA and other relevant regulatory authorities before any of our product candidates can obtain marketing approval. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

The regulatory authorities may, at any time, following approval of a product for sale, audit the manufacturing facilities for such product or institute biennial inspections. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon our third-party manufacturers, our collaborators, or us could harm our business, financial condition, results of operations and prospects.

If our third-party manufacturers, our collaborators, or we fail to comply with applicable cGMP regulations, FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be harmed.

Additionally, if supply from any third-party manufacturers is delayed or interrupted, there could be a significant disruption in the supply of our clinical or commercial material. We have agreements in place with our contract manufacturers pursuant to which we are collaborating on cGMP manufacturing processes and analytical methods for the manufacture of our AAV product candidates. Therefore, if we are unable to enter into an agreement with our contract manufacturers to manufacture clinical or commercial material for our product programs, or if our agreement with our contract manufacturers were terminated, we would have to find suitable alternative manufacturers. This could delay our or our collaborators' ability to conduct clinical trials or commercialize our current and future product candidates. The regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

Any contamination in the manufacturing process for our products or product candidates, shortages of raw materials, cells or reagents, or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Failure to obtain access to or to protect intellectual property related to the manufacturing of our products or product candidates may result in changes, delays and/or inability to manufacture such products or product candidates.

The intellectual property related to the manufacture of biological products is complex. If we are unable to maintain control of manufacturing technology such as our trade secrets, or we are unable to protect ongoing improvements comprehensively and in a sufficient number of jurisdictions, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

We presently manufacture our product candidates using either an insect cell AAV production system or a mammalian cell system. We are aware of third parties which also use these systems in the manufacture of their products and who hold intellectual property on their AAV manufacturing systems. If we determine that access to certain third-party intellectual property is necessary for the manufacturing of our products and product candidates and are unable to license or otherwise access this intellectual property, it would impact our ability to produce products for commercial sale or product candidates for preclinical testing or clinical trials and our development timelines and operations timelines could be adversely affected.

Risks Related to Our Business Operations

We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity, or for which there is a greater likelihood of success.

The success of our business depends upon our ability to identify, develop and commercialize product candidates generated through our gene therapy and vectorized antibody platforms. Research programs to identify new product candidates require substantial technical, financial and human resources. Our product candidates are in preclinical development. To date, our research and development efforts have focused on our VY-AADC (NBIb-1817) and VY-HTT01 programs. We have terminated our VY-AADC program, and we deprioritized our VY-HTT01 program as we have decided to develop a second-generation product candidate for the treatment of Huntington's disease. Our current portfolio of product candidates is subject to change as we continue to conduct preclinical testing and to develop product candidates and prioritize or abandon product candidates based on such results and other factors. For example, in August 2022, we announced a re-prioritization of our portfolio based on a review evaluating our programs based on, among other things, our assessment of their potential for competitive differentiation, the efficiency of such product candidate's path to human proof of biology or proof of mechanism (reflecting the availability of validated biomarkers), unmet medical need, commercial opportunity, and alignment with our overall strategy, as well as supportive preclinical data. We may also fail to identify other product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Similar to our prior investments with regard to our VY-AADC (NBIb-1817) and VY-HTT01 program, our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, option and license, or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. Several of our current preclinical programs have previously been part of collaborations with third parties. While we have invested significant resources in these programs, we may decide in the future to cease development activities on one or more of them.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could harm our business, financial condition, results of operations and prospects.

Our future success depends on our ability to retain key members of our management and research and development teams, and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, technical, and scientific expertise of principal members of our management, scientific, and clinical teams. While we have entered into employment agreements or offer letters with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. We currently do not have "key person" insurance on any of our employees. The loss of the services of one or more of our current employees might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified employees, consultants and advisors for our business, including scientific and technical personnel, is critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology

companies and academic institutions for individuals with similar skill sets. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval, the termination of relationships with collaborators, and the reduction of our workforce in connection with the development of a new portfolio and platform strategy may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of, certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and could harm our business, financial condition, results of operations and prospects.

If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. We can provide no assurances that we will have sufficient resources in the future to manage all of our planned programs. Future growth would impose significant added responsibilities on members of management, may lead to significant added costs, and may divert our management and business development resources. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants, collaborators, and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Current and future legislation may increase the difficulty and cost for us and any collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any collaborators, to profitably sell any products for which we obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in

additional downward pressure on the price that we, or any collaborators, may receive for any approved products. If reimbursement of our products is unavailable or limited in scope, our business could be materially harmed.

In March 2010, President Obama signed the ACA into law. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2030 under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit affirmed the lower court's ruling that the individual mandate portion of the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On June 17, 2021, the U.S. Supreme Court dismissed this case after finding that plaintiffs do not have standing to challenge the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our drug products, if and when approved.

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States and other jurisdictions. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, former President Trump issued several executive orders intended to lower the costs of prescription drug products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other

economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, the Centers for Medicare & Medicaid Services, or CMS, issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, the Department of Health and Human Services, or the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. The final rule is currently the subject of ongoing litigation, but at least six states (Vermont, Colorado, Florida, Maine, New Mexico, and New Hampshire) have passed laws allowing for the importation of drugs from Canada with the intent of developing SIPs for review and approval by the FDA. Further, in November 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1, 2022, but with passage of the Inflation Reduction Act has been delayed by Congress to January 1, 2032.

In July 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. The order directed the HHS to create a plan within 45 days to combat "excessive pricing of prescription pharmaceuticals and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such pharmaceuticals, and to address the recurrent problem of price gouging." In September 2021, the HHS released its plan to reduce pharmaceutical prices. The key features of that plan are to: (a) make pharmaceutical prices more affordable and equitable for all consumers and throughout the health care system by supporting pharmaceutical price negotiations with manufacturers; (b) improve and promote competition throughout the prescription pharmaceutical industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments.

More recently, on August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products became the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on any drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay

rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year. In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, health care organizations and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

In other countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or our collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our products or product candidates to other available therapies. If reimbursement of our products or product candidates is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

These measures, as well as others adopted in the future, may result in additional downward pressure on the price that we receive for any approved product we or our collaborators might bring to market. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from that we, or our collaborators, may successfully develop and for which we, or they, may obtain marketing approval and may affect our overall financial condition and ability to develop or commercialize product candidates.

We may be subject, directly or indirectly, to federal, state, and foreign healthcare laws and regulations, including fraud and abuse laws and false claims laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims act, and the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed research and development, sales, marketing and educational programs. In addition, we may be subject to data privacy laws by both the federal government and the states in which we conduct our

business. Such laws that may constrain the business or financial arrangements and relationships through which we conduct our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for either the referral of an individual for, or the purchase, recommendation, leasing or furnishing of, an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. Further, the ACA amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;

- the federal civil and criminal false claims laws and civil monetary penalty laws, including the civil False Claims Act, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties. The ACA provided and recent government cases against pharmaceutical and medical device manufacturers support the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the civil False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing or attempting to execute a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

- federal transparency laws, including the federal Physician Payments Sunshine Act, which is part of the ACA, that requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to payments and other transfers of value provided to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members, by the 90th day of each subsequent calendar year, and disclosure of such information is made by CMS on a publicly available website; and

- state and/or foreign law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws that may apply to arrangements and claims involving health care items or services reimbursed by non-governmental third-party payors; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union and other countries. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws such as laws of individual European Union Member States or the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, and contractual obligations or our failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, European Union, and United Kingdom. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, costly changes to our business practices, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information that we may obtain directly or indirectly from health care providers, health plans or other health care industry stakeholders and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether we handle protected health information and whether it has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on certain businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the European Union's General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA

prescribes significant penalties for companies that violate its requirements. On November 3, 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The CPRA may apply to some of our business activities. In addition, other states, including Connecticut, Colorado, Utah, and Virginia, have recently passed state privacy laws; Virginia's law became effective January 1, 2023, and the laws in the other three states are scheduled to go into effect later in 2023. Congress, at the federal level, and other states are expected to consider similar laws in the future. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR has been and will continue to require a rigorous and time-intensive process that has increased and will continue to increase our cost of doing business or require us to change our business practices. Despite those efforts, there is a risk that we or our collaborators may be subject to fines and penalties, litigation, and reputational harm in connection with any activities occurring in the European Union, which could adversely affect our business, prospects, financial condition and results of operations.

GDPR restrictions on transfers of personal data from the European Union to the United States are unsettled and may impact our business operations. The GDPR generally prohibits transfers of personal data of European Union data subjects outside of the European Union, unless a lawful data transfer solution has been implemented or a specific exception applies. In July 2020, the European Court of Justice invalidated the Privacy Shield program, a voluntary self-certification privacy protection mechanism that facilitated transfers of personal data from the European Union to the United States. The court upheld the validity of an alternative contractual mechanism for such data transfers but required companies to take additional steps, such as evaluating supplementary measures that may need to be taken to protect the transferred personal data. In October 2022, President Biden signed an executive order to implement the European Union -U.S. Data Privacy Framework, which would replace the Privacy Shield. In December 2022, the European Commission began the European Union's process for adopting the European Union-U.S. Data Privacy Framework, but it is unclear if and when the framework will be finalized and whether it will be challenged in court. Continued uncertainty relating to European Union - U.S. data transfers may adversely impact our business operations in the European Union.

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. Following the exit of the United Kingdom, or UK, from the European Union, the United Kingdom's the Data Protection Act of 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by GDPR. Privacy and data security laws in several other countries loosely follow GDPR as a model but often contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and any eventual commercialization and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions. Any failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to

have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Although we maintain clinical trial liability insurance in the amount of $10.0 million per occurrence and $10.0 million in the aggregate, this insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial. In addition, if we successfully commercialize any product candidate, we will need to obtain product liability insurance. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or from any other work-related injuries, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance and workers' compensation insurance for certain costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could harm our business, financial condition, results of operations and prospects.

Further, with respect to the operations of any current or future collaborators or third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products.

Risks Related to the Commercialization of Our Product Candidates

The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated prevalence range for the indications we are targeting has involved collating limited data from multiple sources. While we believe these sources are reliable, we have not independently verified the data. Accordingly, the prevalence estimates included in our periodic reports and other reports filed with or furnished to the SEC, should be viewed with caution. Further, the data and statistical information used in such reports, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties, and such data is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business. Additionally, because some patients with the diseases we are targeting in the United States, the European Union, and elsewhere may have increased susceptibility to COVID-19, the COVID-19 pandemic could limit the number of patients willing to participate in clinical trials related to our products or amenable to treatment with our products, which would harm our results of operations and our business.

If we are unable to establish sales, medical affairs and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any product revenue.

To successfully commercialize any products that may result from our clinical development programs, we will need to further develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

Under the 2019 Neurocrine Collaboration Agreement, Neurocrine agreed to fund the clinical development through the readout of the RESTORE-1 Phase 2 clinical trial for VY-AADC (NBIb-1817). If Neurocrine had not terminated the 2019 Neurocrine Collaboration Agreement with respect to VY-AADC (NBIb-1817), after the data readout of the RESTORE-1 Phase 2 clinical trial, we would have had the option to either: (1) co-commercialize VY-

AADC (NBIb-1817) with Neurocrine in the United States under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties based on ex-U.S. sales, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine. Under the terms of the 2019 Neurocrine Collaboration Agreement for the FA Program, Neurocrine has agreed to fund the development through the Phase 1 clinical trial of VY-FXN01. After the data readout of the Phase 1 clinical trial, we have the option to either: (1) co-commercialize VY-FXN01 with Neurocrine in the United States under a 60/40 cost and profit-sharing arrangement, 60% to Neurocrine and 40% to us, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine.]

Under the 2023 Neurocrine Collaboration Agreement, Neurocrine agreed to fund in conducting non-clinical development activities for the GBA1 Program. Upon our receipt of topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program, we will have the option to either: (1) co-commercialize collaboration products in the GBA1 Program with Neurocrine in the United States under a 50/50 cost- and profit-sharing arrangement and receive milestones and royalties based on ex-U.S. sales, or (2) retain the right to receive milestone payments and royalties based on global sales pursuant to the full global commercial rights granted to Neurocrine. In the event the Company exercises its 2023 Co-Co Option, the parties have also agreed that Neurocrine is entitled to receive (in addition to its 50% share of profits) 50% of the Company's share of profits until the Company's obligation to repay 50% of all development costs incurred by Neurocrine in connection with the GBA1 Program prior to such exercise have been paid off out of such 50% of the Company's share of profits. The 2023 Co-Co Trigger Event is the date on which the Company receives topline data from the first Phase 1 clinical trial for a product candidate being developed pursuant to the GBA1 Program.

In the future, we may seek to enter into collaborations regarding other of our product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. We might face unforeseen costs and expenses associated with creating an independent sales and marketing organization. Our sales personnel might also face difficulties obtaining access to physicians or being able to persuade adequate numbers of physicians to use or prescribe our products or selling our products if we lack complementary products, which could disadvantage us compared to companies with more extensive product lines. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients, or third-party payors, we will not be able to generate significant revenues from such product, which could harm our business, financial condition, results of operations and prospects.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they receive regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other

third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient and the indication;
- convenient and easy-to-administer compared to alternative treatments;
- cost-effective compared to alternative treatments; and
- neither experimental nor investigational.

No uniform policy requirement for coverage and reimbursement for biopharmaceutical products exists among third-party payors. Therefore, coverage and reimbursement for such products can differ significantly from payor to payor. As a result, obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to each different payor supporting scientific, clinical and cost-effectiveness data, and to receive the support of medical associations and technology assessment committees. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment including our research, development, manufacture, sales, and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement are critical to new product acceptance. Additionally, there may be significant delays in obtaining coverage and reimbursement for newly approved drugs and biologics, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products.

The CMS is responsible for determining whether a product should be approved for coverage and reimbursement under the Medicare program. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for novel products such as ours, as there is no body of established practices and precedents for these types of products. Currently, no gene therapy product has been approved for coverage and reimbursement by the CMS. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage

and reimbursement for our product candidates, especially given that the cost of our product candidates is likely to be very high and pricing of such products is highly uncertain.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug and device products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Therefore, it is difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from the FDA in the United States, EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the support and acceptance of medical associations and technology assessment committees, physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market

acceptance of gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission, or other regulatory authorities;
- patient awareness of, and willingness to seek, genotyping;
- the willingness of physicians to prescribe new therapies;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling or restrictions on the use of our products together with other medications;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Our gene therapy and vectorized antibody approaches utilize vectors derived from viruses that are selectively engineered, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene and vectorized antibody therapies remain novel technologies, with few gene therapy products approved to date in the United States and the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. Medical events such as the COVID-19 pandemic that emphasize harmful effects of certain viruses could also indirectly foster negative public perception of virus-based therapies. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our

product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop.

For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using non-AAV gene therapy vectors. Adverse events and SAEs in our clinical trials such as the MRI abnormalities detected in some patients dosed in the RESTORE-1 Phase 2 clinical trial, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

If we obtain approval to commercialize our product candidates outside of the United States, in particular in the United Kingdom or European Union, a variety of risks associated with international operations could harm our business.

We expect that we will be subject to additional risks in commercializing our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced or loss of protection under our intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism, from natural disasters including earthquakes, typhoons, floods and fires, or from economic, social, or political instability; and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

We must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of

internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In many foreign countries, it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the FCPA. In addition, the FCPA presents particular challenges in the pharmaceutical industry because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, we will be required to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Although we expect to implement policies and procedures designed to comply with these laws and policies, there can be no assurance that our employees, contractors and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Risks Related to Our Intellectual Property

Our rights to develop and commercialize our product candidates are subject to, in part, the terms and conditions of licenses granted to us by others.

We are reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. These licenses may also require us to grant back certain rights to licensors and to pay certain amounts relating to the use of the licensed intellectual property. For example, The Touchlight License Agreement obligates us to make future milestone and royalty payments if we, or our collaboration partners or TRACER capsid licensees, use a capsid created using certain DNA preparation processes licensed under the Touchlight License Agreement.

In some circumstances, particularly in-licenses with academic institutions, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce or defend the patents, covering technology that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be adversely affected. In certain circumstances, we have or may license technology from third parties on a non-exclusive basis. In such instances, other licensees may have the right to enforce our licensed patents in their respective fields, without our oversight or control. Those other licensees may choose to enforce our licensed patents in a way that harms our interest, for example, by advocating for claim interpretations or agreeing on invalidity positions that conflict with our positions or our interest. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we own or may own in the future.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly diligence obligations in developing and commercializing the product. Certain of our license agreements contain "no challenge" clauses which preclude and prevent us from taking any action to limit or narrow the intellectual property of a licensor. In some cases, these limitations extend to any intellectual property of our licensor and not just that which is licensed to us. Such constraints may limit our ability to develop or commercialize products or to expand such efforts beyond the scope of any license. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

If we fail to comply with our obligations under these license agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to develop, manufacture, or market products covered by the license or may face other penalties under the agreements. Termination of any of our agreements involving intellectual property or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Termination may also result in unfavorable terms associated with such termination or may result in obligations on our part to license or grant back intellectual property rights to prior licensors.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive, royalty-free license authorizing the U.S. government, or a third party on its behalf, to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The U.S. government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government, or a third party on its behalf, of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

Our success depends, in large part, on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates and manufacturing technology. We and our licensors have sought, and we intend to seek in the future, to protect our proprietary position by filing patent applications in the United States and abroad related to many of our technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not have and may not in the future be able to file, prosecute, maintain, enforce, defend or license all necessary or desirable patent applications in some or all relevant jurisdictions at a reasonable cost or in a timely manner. For example, in some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we may not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. In some cases, we may be able to obtain patent protection, but such protections may expire before we commercialize the product protected by those rights, leaving us no meaningful protection for our products. In other cases, where our intellectual property is being managed by a third-party collaborator, licensee or partner, that third party may fail to act diligently in prosecuting, maintaining, defending or enforcing our patents. Such conduct may result in the failure to maintain or obtain protections, loss of rights, loss of patent term or, in cases where a third party has acted negligently or inequitably, patents being found unenforceable.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value, narrow the scope, or eliminate the enforceability of our and our licensors' patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, only upon issuance or not at all. Therefore, we cannot be certain that we, or a licensor, were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, respectively, or which entity was the first to file for patent protection until such patent application publishes or issues as a patent. Databases for patents and publications, and methods for searching them, are inherently limited, so it is not practical to review and know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensed patent rights are uncertain.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

In spite of a legal presumption of validity, the issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity, or enforceability which may be challenged in the courts and patent offices in the United

States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology, resulting in termination of our access to such intellectual property, or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, result in loss of access, or increase what we believe to be our financial or other obligations under the relevant agreement, any of which could harm our business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to certain intellectual property, through licenses from third parties, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical or technical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We currently co-own certain intellectual property rights with one or more third parties. We may not be able to obtain a license to the third parties' interest such that we have exclusive access and control of such co-owned assets. In this case, and depending on the jurisdiction of the patent filing, we may not be able to license, enforce, or exploit the co-owned rights without the consent from, or an accounting to, the other co-owners.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to develop our program. We may also decide not to exercise an option to such institutional rights.

If we decide not to obtain, or are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office, or USPTO, and various government patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own in the future. We rely on our outside counsel or our licensors to pay these fees due to patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction, and may compromise the strength of other intellectual property in our portfolio. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

On February 1, 2019 the government of Venezuela, in response to certain U.S. sanctions, began to require that foreign entities pay all official fees, including patent fees (either for pending matters or new petitions), in PETRO, a "cryptocurrency" created by the Nicolás Maduro administration in February 2018 as a way to collect U.S. dollars while avoiding American financial sanctions issued under an Executive Order of President Trump on March 19, 2018. The Executive Order banned transactions involving "any digital currency, digital coin, or digital token, that was issued by, for, or on behalf of the Government of Venezuela on or after January 9, 2018." The prohibition is applicable to any U.S. entity unless exempted by license. We do not hold such a license and therefore may not be able to secure patents in Venezuela.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive. Our intellectual property rights may vary from country to country and foreign protections could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products or methods of treatment, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. For example, an April 2022 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. With Brexit, there is uncertainty associated with obtaining, defending, and enforcing intellectual property rights in the United Kingdom. International treaties and regulations promulgated as a result of this transition could impede or eliminate our ability to obtain or maintain meaningful intellectual property rights in the United Kingdom. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we

initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Issued patents covering our technology or product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensees or licensors or we initiate legal proceedings against a third party to enforce a patent covering our technology or one of our product candidates, the defendant could counterclaim that the patent covering such technology or product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that an individual connected with prosecution of the patent, including an inventor, an employee of the company, a collaborator or advisor, withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include pre-issuance submissions, *ex parte* re-examination, post-grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Some of these mechanisms may even be exploited anonymously by third parties. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our technology or product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensees or licensors were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose part or, all of the patent protection on one or more of our product candidates or our supporting technology. Such a loss of patent protection could harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection, nondisclosure, and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, collaborators, contractors, and other third parties. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or threatened with, infringement litigation claims regarding our products and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including *ex parte* re-examination, post-grant review and *inter partes* review before the USPTO or foreign patent offices. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of the merit of the claim.

In November 2022, we and Touchlight entered into the Touchlight License Agreement to allow for our historical use of a certain DNA preparation process, or the Subject DNA Preparation Process, and to authorize the prospective exploitation of TRACER capsids that we have previously created using the Subject DNA Preparation Process. As previously referenced in the Risk Factor section of our prior periodic reports, Touchlight had made us aware earlier in 2022 that it believed its intellectual property rights could potentially be asserted against us, although we disagreed with this assessment. In connection with entering into the Touchlight License Agreement, Touchlight also agreed to release any potential claims against us regarding the alleged historical use of certain of Touchlight's intellectual property rights and exploitation of TRACER capsids created with the alleged use of such intellectual property rights.

Potential parties may emerge and choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such asserted third-party U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similar challenges exist in other jurisdictions. If we are found to infringe a third-party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals, and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, reputation, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may infringe our intellectual property rights or the intellectual property rights of our licensees or licensors, or we may be required to defend against claims of infringement. To counter infringement or unauthorized use claims or to defend against claims of infringement can be expensive and time consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our directors, employees, consultants, and advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that these individuals do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees, consultants, advisors and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a "first-to-invent" system to a "first-inventor-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-inventor-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO has promulgated regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-inventor-to-file provisions, became effective on March 16, 2013. The Leahy-Smith Act has resulted in an increased investment in filing applications earlier, and consequently has increased the uncertainties and costs surrounding the prosecution of our patent applications, and may increase the enforcement or defense of our issued patents, all of which could harm our business, financial condition, results of operations and prospects.

The administrative tribunal created by the Leahy-Smith Act, known as the Patent Trial and Appeals Board, or PTAB, may have an impact on the operation of our business in the future. For example, the initial results of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many U.S. patent claims. The availability of the PTAB as a lower-cost, faster and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own licensed patents will be challenged, thereby increasing the uncertainties and costs of maintaining and enforcing them. Moreover, if such challenges occur, we may not have the right to control the defense. In certain situations, we may be required to rely on our licensor to consider our suggestions and to defend such challenges, with the possibility that it may not do so in a way that best protects our interests.

We also may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in other contested proceedings such as opposition, derivation, reexamination, *inter partes* review, or post-grant review proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future products.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain as the courts address issues such as patenting genes or gene products. Recent guidance provided under *Berkheimer v HP, Inc*. (April 19, 2018) and *Vanda Pharmaceuticals, Inc. v West-Ward Pharmaceuticals* (June 7, 2018) instruct USPTO examiners on the ramifications of the court rulings as applied to method of treatment claims, natural products and principles including all naturally occurring nucleic acids. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, the recent USPTO guidance could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the court decisions referenced above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact decisions from the U.S. Supreme Court's decisions in *Mayo Collaborative Services v. Prometheus Laboratories* and *Molecular Pathology v. Myriad Genetics, Inc.* or other applicable court decisions may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have an adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Moreover, although the U.S. Supreme Court has held that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

Outside the United States, other courts have also begun to address the patenting of genetic material. In August 2015, the Australian High Court ruled that isolated genes cannot be patented in Australia. The decision did not address methods of using genetic material. Any ruling of a similar scope in other countries could affect the scope of our intellectual property rights. The ambiguities and changing law in all countries as to patenting genetic material may directly affect our ability to secure and/or maintain patent protection for our products.

If we do not obtain patent term extension and regulatory exclusivity for our product candidates, our business may be harmed.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents, which may cover non-gene therapy compounds, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. The

Hatch-Waxman Act permits a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended per FDA-approved product, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Further, certain of our licenses currently or in the future may not provide us with the right to control decisions the licensor or its other licensees on Orange Book listings or patent term extension decisions under the Hatch-Waxman Act. Thus, if one of our important licensed patents is eligible for a patent term extension under the Hatch-Waxman Act, and it covers a product of another licensee in addition to our own product candidate, we may not be able to obtain that extension if the other licensee seeks and obtains that extension first. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements.

The BPCIA provides up to 12 years of market exclusivity for a reference biological product. We may not be able to obtain such exclusivity for our products. Moreover, the applicable time-period or the scope of patent protection afforded during any such extension could be less than we request. If we are unable to obtain patent term extension or the scope of term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be materially reduced.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and such rights may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we own, license or may access in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and

- we may choose not to file a patent for certain inventions, trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

We may not be able to maintain sufficient control over our proprietary know-how or trade secrets when employees, consultants, advisors or persons with access to our proprietary information terminate their relationship with us.

Despite our efforts to protect our proprietary know-how and trade secrets, our competitors may discover this information, or obtain the benefit of this information, through a breach of confidentiality and/or non-competition obligations by persons who were formerly associated with us but who have established relationships as employees, contractors, consultants or advisors with other companies, including our competitors. The recent departures of certain executives, key employees, consultants or advisors, and the restructuring of our organization, may make it more difficult to enforce our rights in protecting this information. Further, if discovered in a timely manner, our efforts to enforce rights to protect against these types of breaches may not be possible under law, or may not be successful if commenced.

It is also possible that, as we grow and establish ourselves in multiple geographic areas, alignment and/or compliance with company polices may not be consistently maintained. In any such cases, the risk of loss of control or proper management of our proprietary information could jeopardize our intellectual property.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our product candidates and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our gene therapy and vectorized antibody platforms and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

Risks Related to Ownership of Our Common Stock

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Persons who were our stockholders prior to our initial public offering continue to hold a substantial number of shares of our common stock. If such persons sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline.

In addition, shares of common stock that are either subject to outstanding options or restricted stock units, or RSUs, or reserved for future issuance under our stock incentive plans will become eligible for sale in the public market

to the extent permitted by the provisions of various vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended. We have also filed registration statements on Form S-8 permitting shares of common stock issued on exercise of options or the settlement of RSUs to be freely sold in the public market. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. We also have an effective registration statement on Form S-3 for the sale of up to $300.0 million in aggregate of an indeterminate number of shares of common stock and preferred stock, an indeterminate principal amount of debt securities, and an indeterminate number of depositary shares, subscription rights, warrants, purchase contract and units, of which we have reserved $75.0 million for the offering, issuance, and sale of common stock through at-the-market offerings or negotiated transactions under a sales agreement we entered into with Cowen and Company, LLC, on November 8, 2022.

Certain holders of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.

The price of our common stock is likely to be volatile and may fluctuate substantially. From January 1, 2022 through December 31, 2022, the sales price of our common stock ranged from a high of $10.60 to a low of $2.60 on the Nasdaq Global Select Market. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- our success in commercializing any product candidates for which we obtain marketing approval;
- regulatory action and results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- the results of clinical trials of our product candidates;
- the results of clinical trials of product candidates of our competitors;
- the commencement, termination, and success of our collaborations, including the ability or willingness of our collaboration partners to fulfill their obligations to us;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or technologies, the cost of commercializing such product candidates, and the cost of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;

- the ability to secure third-party reimbursement for our product candidates;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- other factors described in this "Risk Factors" section and elsewhere in this Annual Report on Form 10-K.

If our operating results fall below the expectations of investors or securities analysts for a given period, the price of our common stock could decline substantially. Furthermore, any fluctuations in our operating results from period to period may, in turn, cause the price of our stock to fluctuate substantially. We believe that such comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. We also may face securities class-action litigation if we cannot obtain regulatory approvals for or if we otherwise fail to commercialize our product candidates. We and certain of our current and former officers and directors were previously named as defendants in a purported class action lawsuit. This proceeding and other similar litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

We have broad discretion in how we apply our available funds, and we may not use these funds effectively, which could affect our results of operations and cause our stock price to decline.

Our management will have broad discretion in the application of our existing cash, cash equivalents and marketable securities and could spend these funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply our available funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates and preclinical programs. Pending their use, we may invest our available funds in a manner that does not produce income or that loses value.

We are a "smaller reporting company" and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to qualify as a smaller reporting company if we have (a) a non-affiliate public float in excess of $250 million and annual revenues in excess of $100 million during our last fiscal year, or (b) a non-affiliate public float in excess of $700 million, in each case determined on an annual basis as of the last business day of our second quarter. As a smaller reporting company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies. These exemptions include:

- being permitted to provide only two years of audited consolidated financial statements in this Annual Report on Form 10-K, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- reduced disclosure obligations regarding executive compensation; and
- not being required to furnish a stock performance graph in our annual report.

We expect to take advantage of some or all of the available exemptions until we cease to be a smaller reporting company. We may cease to qualify as a smaller reporting company as early as June 30, 2023, which would require us to

comply with disclosure requirements that are applicable to other public companies that are not smaller reporting companies following the filing of our Annual Report on Form 10-K for the year ending December 31, 2023, and any portions of our definitive proxy statement relating to our 2024 Annual Meeting of Stockholders incorporated by reference therein. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We have recently been, and could in the future be, subject to legal actions and proceedings related to the decline in our stock price, which could distract our management and could result in substantial costs or large judgments against us.

The market prices of securities of companies in the biotechnology and pharmaceutical industry, including the market price of our common stock, have been extremely volatile and have experienced fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. On January 22, 2021, a putative class action lawsuit was filed in the U.S. District Court for the Eastern District of New York (later transferred to the U.S. District Court for the District of Massachusetts) against us and certain of our current and former officers and directors. The complaint sought, among other things, unspecified compensatory damages, interest, attorneys' and expert fees and costs. On July 2, 2021, the lead plaintiff voluntarily dismissed the action without prejudice against all defendants and as to all claims, and this action is no longer pending. Nonetheless, due to the volatility in, or the unfulfilled expectations of stockholders for, our stock price, we may be the target of similar litigation in the future.

In connection with such legal proceedings, we could incur substantial costs and such costs and any related settlements or judgments may not be covered by insurance. We could also suffer an adverse impact on our reputation and a diversion of management's attention and resources, which could cause serious harm to our business, operating results and financial condition.

Provisions in our amended and restated certificate of incorporation and bylaws and Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of members of the board is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;

- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our amended and restated certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (a) any derivative action or proceeding brought on our behalf, (b) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (c) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws or (d) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision is inapplicable to actions arising under the Securities Exchange Act of 1934, as amended, and we likewise do not intend to apply this choice of forum provision to actions arising under the Securities Act of 1933, as amended.

This choice of forum provision may limit a stockholder's ability to bring a claim that is not arising under the Securities Exchange Act of 1934, as amended, or the Securities Act of 1933, as amended, in a judicial forum that he, she or it finds favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs and business interruption that could have a material adverse effect on our business, financial condition or results of operations.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

General Risk Factors

We might not be able to utilize a significant portion of our net operating loss carryforwards.

As of December 31, 2022, we had both federal and state NOL carryforwards of $175.1 million and $166.5 million, respectively, which expire beginning in 2033. These NOL carryforwards could expire unused and be unavailable to offset our future income tax liabilities. As described above under the heading "Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition," the TCJA, as amended by the CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. Nor is it clear how various states will respond to the TCJA, the FFCR Act or the CARES Act. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. Furthermore, the use of NOL carryforwards may become subject to an annual limitation under Section 382 of the Code and similar state provisions in the event of certain cumulative changes in the ownership interest of significant shareholders in excess of 50 percent over a three-year period. This could limit the amount of NOL carryforwards that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of a company immediately prior to the ownership change. Our company has completed several transactions since its inception which resulted in an ownership change under Section 382 of the Code. In addition, future changes in our stock ownership, some of which are outside of our control, could result in ownership changes in the future. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, ransom requests, sabotage, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations or the operations of those third parties with which we contract, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions, and could require a substantial expenditure of resources to remedy. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. We could also be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in our information systems and networks, including personal information of our employees. Outside parties may attempt to penetrate our systems or those of the third parties with which we contract or to fraudulently induce our employees or employees of such third parties to disclose sensitive information to gain access to our data or to use such access to request cash compensation in the form of a ransom for the return of such data.

The number and complexity of these threats continue to increase over time. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Despite our efforts, the possibility of these events occurring cannot be eliminated entirely. Although we maintain cyber risk insurance for certain costs we may incur due to a cyber-related event, this insurance may not provide adequate coverage against potential liabilities. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, or a loss of cash in response to ransom threats, we could incur liability, our competitive and financial position and the market perception of the effectiveness of our security measures could be harmed, our credibility could be damaged, and the further development and commercialization of our product candidates could be delayed.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not applicable.

ITEM 2. PROPERTIES

Our corporate headquarters are located in Cambridge, Massachusetts. Other operations, including laboratory space, are located in Lexington, Massachusetts. We lease our office and laboratory space, which consist of approximately 26,148 square feet located in Cambridge, Massachusetts and 32,142 square feet located in Lexington, Massachusetts.

ITEM 3. LEGAL PROCEEDINGS

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of any such matters cannot be predicted with certainty, as of December 31, 2022, we were not party to any material pending proceedings. No material governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Our common stock has been traded on the Nasdaq Global Select Market under the symbol "VYGR" since November 11, 2015. Prior to this time, there was no public market for our common stock.

Stockholders

As of March 1, 2023, there were approximately 12 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

We have not paid any cash dividends on our common stock since inception and do not anticipate paying cash dividends in the foreseeable future.

Recent Sales of Unregistered Securities

Set forth below is information regarding shares of our common stock issued and stock options granted by us for the twelve months ended December 31, 2022 that were not registered under the Securities Act of 1933, as amended, or the Securities Act and that have not otherwise been described in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

On October 1, 2022, we granted to two executives restricted stock unit awards settleable for an aggregate of 163,000 shares of our common stock. On October 17, 2022, we granted stock options to one new employee to purchase

an aggregate of 60,000 shares of our common stock at an exercise price of $6.01 per share. On November 28, 2022, we granted stock options to one new employee to purchase an aggregate of 54,000 shares of our common stock at an exercise price of $5.57 per share. These options and restricted stock units were made outside of our 2015 Stock Option and Incentive Plan as an inducement material to such individual's acceptance of an offer of employment with us in accordance with Nasdaq Listing Rule 5635(c)(4). We intend to file a registration statement on a Form S-8 to register the shares of common stock underlying these inducement awards prior to the time at which the awards become exercisable or settleable, as applicable.

ITEM 6. RESERVED

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors. We discuss factors that we believe could cause or contribute to these differences below and elsewhere in this report, including those set forth under Item 1A. "Risk Factors" and under "Forward-Looking Statements" in this Annual Report on Form 10-K.

We are a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. We believe the potential of both disciplines has been constrained by delivery challenges; we are leveraging expertise in capsid discovery and neuropharmacology to address these constraints. Our gene therapy platforms enable us to engineer, optimize, manufacture and deliver adeno-associated virus, or AAV, based gene therapies that we believe have the potential to safely provide durable efficacy. Our team of experts in the field of AAV gene therapy and neuroscience first identifies and selects diseases in which we believe an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. We then engineer and optimize an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

We are identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. Our team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood brain barrier, or BBB. The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates. We believe that capsids we discover through our TRACER discovery platform, which we refer to as TRACER capsids, have the potential to significantly enhance the efficacy and safety of our single dose gene therapies, which we expect to be delivered with targeted surgical delivery or systemic infusions, as compared with conventional capsids.

In addition to leveraging TRACER capsids in potential licensing arrangements, we are advancing our own proprietary pipeline of drug candidates for neurological diseases. Our wholly-owned prioritized pipeline programs include: superoxide dismutase 1, or SOD1, gene therapy for amyotrophic lateral sclerosis, or ALS, and an anti-tau antibody for Alzheimer's disease. We have identified a lead development candidate for our anti-tau antibody program and we expect to identify a lead development candidate for our SOD1 program during the first half of 2023. We expect to file INDs for both programs in 2024. In addition to these two wholly-owned programs, we are actively advancing two programs in collaboration with Neurocrine Biosciences, Inc., or Neurocrine: a glucocerebrosidase 1, or GBA1, gene therapy program for Parkinson's disease and other GBA1-mediated diseases, and a FXN gene therapy program for Friedreich's ataxia. We also maintain a robust early research pipeline of wholly-owned and collaborative gene therapy programs for neurological diseases.

We have a history of incurring significant losses. We reported a net loss of $46.4 million for the year ended December 31, 2022. As of December 31, 2022, we had an accumulated deficit of $393.5 million. We reported a net loss of $71.2 million for the year ended December 31, 2021. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct preclinical development activities and initiate investigational new drug, or IND, application-enabling studies and clinical trials in connection with our tau antibody program and our SOD1 ALS gene therapy program;
- continue investing in our gene therapy platform to optimize capsid engineering and payload development, manufacturing, dosing, and delivery techniques by continuing to develop our proprietary antibodies and vectorized antibody platform;
- increase our investment in and support for TRACERTM (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA), our proprietary discovery platform to facilitate the selection of AAV capsids and expand our investment to discover TRACER capsids with broad tropism in central nervous system, or CNS, and other tissues with cell-specific transduction properties for particular therapeutic applications;
- conduct joint research and development under our strategic collaborations for the research, development, and commercialization of certain of our pipeline programs, including our FA Program pursuant to a collaboration with Neurocrine entered into in January 2019, or the 2019 Neurocrine Collaboration Agreement, and our GBA1 gene therapy program pursuant to our collaboration and license agreement with Neurocrine entered into on January 8, 2023, or the 2023 Neurocrine Collaboration Agreement;
- initiate additional preclinical studies and clinical trials for, and continue research and development of, our other programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional diseases for treatment with our AAV gene therapies and develop additional programs or product candidates;
- seek marketing and regulatory approvals for any of our product candidates that successfully complete clinical development;
- maintain, expand, protect and enforce our intellectual property portfolio;
- identify, acquire or in-license other product candidates and technologies;
- expand our operational, financial and management systems and personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- increase our product liability and clinical trial insurance coverage as we expand our clinical trials and commercialization efforts; and
- continue to operate as a public company.

We refer to our collaboration agreement with Neurocrine dated as of January 28, 2019 as the 2019 Neurocrine Collaboration Agreement. We refer to our option and license agreement with Pfizer Inc., or Pfizer, as the Pfizer Agreement, and to our option and license agreement with Novartis Pharma AG, or Novartis, as the Novartis Agreement.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future. For the year ended December 31, 2022, we recognized $40.9 million of collaboration revenue from the Pfizer Agreement and the 2019 Neurocrine Collaboration Agreement. For additional information about our revenue recognition policy, see the section titled "—Critical Accounting Policies and Estimates—Revenue."

For the foreseeable future, we expect substantially all of our revenue will be generated from the 2019 Neurocrine Collaboration Agreement and the 2023 Neurocrine Collaboration Agreement, the Pfizer Agreement, the Novartis Agreement, and any other strategic collaborations and out-licensing arrangements we may enter into in the future. If our development efforts are successful, we may also generate revenue from product sales.

Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our program discovery efforts, and the development of our programs, gene therapy platform, proprietary antibodies, and vectorized antibody platform which include:

- employee-related expenses including salaries, benefits, and stock-based compensation expense;
- costs of funding research performed by third parties that conduct research and development, preclinical activities, manufacturing and production design on our behalf;
- the cost of purchasing laboratory supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation and maintenance expenses;
- the cost of securing and protecting intellectual property rights associated with our research and development activities; and
- fees for maintaining licenses under our third-party licensing agreements.

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing, preclinical studies, and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

Research and development activities are central to our business model. We are in the early stages of development of our product candidates. On August 6, 2021, our board of directors approved a strategic restructuring plan to eliminate a portion of our workforce as part of an initiative to reduce expenses and enhance operations. Our research and development costs have decreased relative to pre-2021 levels as a result of this strategic restructuring, and also due to the reevaluation of our product candidate pipeline, our strategic shift to invest in TRACER capsid development efforts, and our initiation of other cost-saving initiatives. As our development programs progress and as we identify product candidates and initiate preclinical studies and clinical trials, we expect research and development costs to increase. However, at this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses. Our expenses will increase if:

- we are required by the FDA or the European Medicines Agency, or EMA, or other regulatory agencies to redesign or modify trials or studies or to perform trials or studies in addition to those currently expected;
- there are any delays in the receipt of regulatory clearance to begin our planned clinical programs; or
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to patent and corporate matters and fees for accounting and consulting services.

Our general and administrative expenses have decreased relative to pre-2021 levels as a result of our strategic restructuring. As a result of the strategic restructuring, there are decreases including a reduction in personnel costs and fees paid to outside consultants, as well as other cost-saving initiatives including a reduction in facility-related expenditures. As our development programs progress and we identify product candidates and initiate preclinical studies and clinical trials, we expect general and administrative expenses to increase to support these additional research and development activities.

Other Income, Net

Other income, net consists primarily of an employee retention tax credit under the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, and interest income on our marketable securities.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our consolidated financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the financial statements prospectively from the date of change in estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies used in the preparation of our financial statements require the most significant judgments and estimates.
Revenue Recognition – ASC 606

We recognize revenue in accordance with Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 606 *Revenue from Contracts with Customers,* or ASC 606.

We enter into license, option, and collaboration agreements which are within the scope of ASC 606, under which we license or provide options to license certain of our product candidates and, in certain cases, perform research and development. The terms of these arrangements typically include payment of one or more of the following: non-

refundable, upfront fees; reimbursement of research and development costs; development, regulatory and commercial milestone payments; option exercise fees; and royalties on net sales of licensed products.

We estimate the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each arrangement that includes variable consideration, we evaluate the amount of potential payment and the likelihood that the payments will be received. We utilize either the most likely amount method or expected amount method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration which is included in the transaction price may be constrained, and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

Our contracts often include development and regulatory milestone payments which are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within our control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, we re-evaluate the probability of achievement of such development milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues in the period of adjustment. To date, we have not recognized any consideration related to the achievement of development, regulatory, or commercial milestone revenue resulting from any of our collaboration arrangements.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (a) when the related sales occur, or (b) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any consideration related to sales-based royalty revenue resulting from any of our collaboration arrangements.

We allocate the transaction price based on the estimated stand-alone selling price of each of the performance obligations. We must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. We utilize key assumptions to determine the stand-alone selling price for performance obligations, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Additionally, in determining the standalone selling price for material rights, we utilize comparable transactions, industry standards for product development and clinical trial success probabilities and estimates of option exercise likelihood. Variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts we would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

Upfront payments and fees are recorded as contract liabilities within deferred revenue upon receipt or when due until we perform our obligations under these arrangements. Amounts are recorded as accounts receivable when our rights to consideration are unconditional. A significant portion of revenue recognized from the 2019 Neurocrine Collaboration Agreement is related to performance obligations pursuant to which revenue is recognized using a proportional performance model. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs. We estimate the expected remaining costs to complete the research and development services for each performance obligation. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure and related

revenue recognition. Changes in our estimates of the expected remaining costs to complete the research and development services for our performance obligations, such as the significant change that occurred in the fourth quarter of 2021 as a result of decisions made by the JSC for the 2019 Neurocrine Collaboration Agreement, can result in significant changes to the amount of revenue we recognize each period.

Results of Operations

Comparison of the years ended December 31, 2022 and 2021:

The following table summarizes our results of operations for the years ended December 31, 2022 and 2021, respectively, together with the changes in those items in dollars:

	Year ended December 31,		
	2022	2021	Change
		(in thousands)	
Collaboration revenue	$ 40,907	$ 37,415	$ 3,492
Operating expenses:			
Research and development	60,764	73,787	(13,023)
General and administrative	30,980	37,246	(6,266)
Total operating expenses	91,744	111,033	(19,289)
Other income, net:			
Interest income (expense)	1,792	(390)	2,182
Other income	2,653	2,811	(158)
Total other income, net	4,445	2,421	2,024
Loss before income taxes	(46,392)	(71,197)	24,805
Income tax provision	16	—	16
Net loss	$ (46,408)	$ (71,197)	$ 24,789

Collaboration Revenue

Collaboration revenue was $40.9 million for the year ended December 31, 2022, and $37.4 million for the year ended December 31, 2021. The increase in collaboration revenue was largely a result of revenue recognized in connection with Pfizer's decision to exercise the first material right for the option to receive an exclusive license, or the Pfizer License Option, along with the expiration of the second material right associated with the Pfizer License Option. This resulted in total revenue recognized of $40.0 million from Pfizer during the year ended December 31, 2022. The increase in collaboration revenue is partially offset by decreased revenue recognized under the 2019 Neurocrine Collaboration Agreement during the year ended December 31, 2022. During the fourth quarter of 2021, we recorded significant revenue associated with a change in estimate of the expected remaining costs to complete the research and development services for our performance obligations under the 2019 Neurocrine Collaboration Agreement, resulting in a significant decrease in revenue to be recorded in future periods. During the year ended December 31, 2021, collaboration revenue was entirely related to research services and cost reimbursement from the 2019 Neurocrine Collaboration Agreement. Our collaboration revenues were not materially impacted by the COVID-19 pandemic during the year ended December 31, 2022.

Research and Development Expense

Research and development expense decreased by $13.0 million from $73.8 million for the year ended December 31, 2021 to $60.8 million for the year ended December 31, 2022. The following table summarizes our research and development expenses for the years ended December 31, 2022 and 2021:

	Year ended December 31,		
	2022	2021	Change
		(in thousands)	
Employee and consultant	$ 29,209	$ 36,385	$ (7,176)
External research and development	15,679	18,486	(2,807)
Facilities and other	7,863	9,483	(1,620)
Professional fees	8,014	9,433	(1,420)
Total research and development expenses	$ 60,764	$ 73,787	$ (13,023)

The decrease in research and development expense for the year ended December 31, 2022 was primarily attributable to the following:

- approximately $7.2 million for decreased compensation costs and stock-based compensation costs associated with lower headcount in research and development functions compared to prior year;
- approximately $2.8 million for decreased external research and development costs primarily related to a reduction in clinical and manufacturing activities to prepare for the first-in-humans trial of the VY-HTT01 for Huntington's disease and a reduction in external costs incurred in connection with the 2019 Neurocrine Collaboration Agreement, partially offset by the technology access fee for our license agreement with Touchlight IP Limited, or the Touchlight License Agreement;
- approximately $1.6 million of decreased facility costs primarily related to the termination of the lease for office and laboratory space at 75 Sidney Street during the second quarter of 2022; and
- approximately $1.4 million for decreased professional fees and related expenses to support the pipeline programs.

General and Administrative Expense

General and administrative expense decreased by $6.3 million from $37.2 million for the year ended December 31, 2021 to $31.0 million for the year ended December 31, 2022. The decrease in general and administrative expense was primarily attributable to the following:

- approximately $3.1 million for decreased compensation costs and stock-based compensation costs associated with lower headcount in general and administrative functions as compared to prior year;
- approximately $1.6 million for decreased facility and other costs primarily related to the termination of the lease for office and laboratory space at 75 Sidney Street during the second quarter of 2022; and
- approximately $1.5 million for decreased legal costs and intellectual property related expenses.

Other Income, Net

Other income, net of approximately $4.4 million was recognized during the year ended December 31, 2022, as compared to $2.4 million during the year ended 2021. Other income, net during the year ended December 31, 2022 primarily related to an employee retention tax credit under the CARES Act and interest income on marketable securities

balances, while other income, net during the year ended December 31, 2021 primarily related to interest income on marketable securities balances.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations primarily through private placements of redeemable convertible preferred stock, public offerings of our common stock, strategic collaborations and option and license arrangements, including our 2019 Neurocrine Agreement and 2023 Neurocrine Collaboration Agreement, our ongoing option and license arrangements with Pfizer and Novartis under the Pfizer Agreement and the Novartis Agreement, respectively, and with our prior collaboration agreements.

As of December 31, 2022, we had cash, cash equivalents, and marketable securities of $118.8 million. Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2022, together with the upfront payment received in February 2023 in connection with the 2023 Neurocrine Collaboration Agreement, along with amounts expected to be received as reimbursement for development costs under our 2019 Neurocrine Agreement and 2023 Neurocrine Collaboration Agreement, will enable us to meet our planned operating expenses and capital expenditure requirements into 2025.

Cash Flows

The following table provides information regarding our cash flows for the years ended December 31, 2022, 2021, and 2020.

	Year ended December 31,		
	2022	2021	2020
		(in thousands)	
Net cash (used in) provided by:			
Operating activities	$ (12,509)	$ (53,525)	$ (96,716)
Investing activities	(7,339)	65,906	112,995
Financing activities	1,110	612	3,163
Net (decrease) increase in cash, cash equivalents, and restricted cash	$ (18,738)	$ 12,993	$ 19,442

Cash Flows from Operating Activities

Net cash used in operating activities was $12.5 million during the year ended December 31, 2022. The cash used in operating activities for the year ended December 31, 2022 was primarily driven by operating expenses, net of stock-based compensation and depreciation, offset by an increase in deferred revenue partially driven by the upfront payment of $54.0 million from Novartis during the year ended December 31, 2022.

Net cash used in operating activities was $53.5 million during the year ended December 31, 2021. The cash used in operating activities for the year ended December 31, 2021 was primarily driven by operating expenses, net of stock-based compensation and depreciation. We also received an upfront payment of $30.0 million pursuant to the Pfizer Agreement.

Net cash used in operating activities was $96.7 million during the year ended December 31, 2020. The cash used in operating activities for the year ended December 31, 2020 was primarily driven by the one-time recognition of $105.2 million deferred revenue related to the termination of the AbbVie Tau Collaboration and the AbbVie Alpha-Synuclein Collaboration, offset by $36.7 million of net income, and changes in working capital.

Cash Flows from Investing Activities

Net cash used in investing activities was $7.3 million during the year ended December 31, 2022. The cash used in investing activities for the year ended December 31, 2022 was primarily due to $54.8 million for purchases of marketable securities and $2.5 million for purchases of property and equipment, offset by $50.0 million from proceeds from maturities and sales of marketable securities.

Net cash provided by investing activities was $65.9 million during the year ended December 31, 2021. The cash provided by investing activities for the year ended December 31, 2021 was primarily due to $70.0 million from maturities of marketable securities and $12.6 million from proceeds of sales of marketable securities partially offset by $15.1 million for purchases of marketable securities and $1.6 million for purchases of property and equipment.

Net cash provided by investing activities was $113.0 million during the year ended December 31, 2020. The cash provided by investing activities for the year ended December 31, 2020 was primarily due to proceeds from maturities of marketable securities of $195.5 million, offset by purchases of marketable securities of $70.4 million and purchases of property and equipment of $12.1 million.

Cash Flows from Financing Activities

Net cash provided by financing activities was $1.1 million during the year ended December 31, 2022 primarily due to the proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Net cash provided by financing activities was $0.6 million during the year ended December 31, 2021 primarily due to the proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Net cash provided by financing activities was $3.2 million during the year ended December 31, 2020 primarily due to the proceeds from the exercise of stock options, and purchases by our employees of our common stock under our employee stock purchase plan.

Funding Requirements

Our expenses decreased during the year ended December 31, 2022 as compared with the prior year as a result of our strategic restructuring, the reevaluation of our product candidate pipeline, our strategic shift to invest in TRACER capsid development efforts, and our initiation of other cost-saving initiatives. We expect our expenses to increase in the longer term, however, as we continue the research and development of, conduct clinical trials of, and seek marketing approval for, our product candidates and as we continue to enter into or conduct activities in connection with our collaboration agreements. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we expect to incur increasing costs associated with operating as a public company, executing financial statement controls, satisfying regulatory and quality standards, fulfilling healthcare compliance requirements, and maintaining product, clinical trial and directors' and officers' liability insurance coverage. We also anticipate the cost of goods and services and the levels of compensation paid to employee will increase due to inflationary conditions existing in the general economy. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital or enter into business development transactions when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Based upon our current operating plan, we expect that our existing cash, cash equivalents, and marketable securities at December 31, 2022, together with the upfront payment received in February 2023 in connection with the 2023 Neurocrine Collaboration Agreement, along with amounts expected to be received as reimbursement for development costs under our 2019 Neurocrine Agreement and 2023 Neurocrine Collaboration Agreement, will enable us

to meet our planned operating expenses and capital expenditure requirements into 2025. Our future capital requirements will depend on many factors, including:

- the scope, progress, results, and costs of product discovery, preclinical studies and clinical trials for our product candidates;
- the scope, progress, results, costs, prioritization, and number of our research and development programs;
- the progress and status of our strategic collaborations and option and license agreements and any similar arrangement we may enter into in the future, including any research and development costs for which we are responsible, and our receipt of any future milestone payments and royalties from our collaboration partners or licensors;
- the extent to which we are obligated to reimburse preclinical development and clinical trial costs, or the achievement of milestones or occurrence of other developments that trigger milestone and royalty payments, under any collaboration or license agreements to which we might become a party, such as the Touchlight License Agreement;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaboration, distribution, or other marketing arrangements for our product candidates on favorable terms, if at all;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies, including any intellectual property associated with such candidates or technologies, acquire or invest in other businesses, or out-license our product candidates, capsids or other technologies;
- the costs of advancing our manufacturing capabilities and securing manufacturing arrangements for pre-commercial and commercial production;
- the level of product sales by us or our collaborators from any product candidates for which we obtain marketing approval in the future;
- the costs of operating as a public company and maintaining adequate product, clinical trial, and directors' and officers' liability insurance coverage; and
- the costs of establishing or contracting for sales, manufacturing, marketing, distribution, and other commercialization capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete. We may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestone payments or royalty payments under our collaboration agreements, will be derived from sales of products that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing and business development transactions to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate product revenues sufficient to achieve consistent profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and option and license arrangements. We do not have any committed external source of funds other than the

amounts we are entitled to receive from our collaboration partners and licensors for reimbursement of certain research and development expenses, potential option exercises, the achievement of specified regulatory and commercial milestones, and royalty payments under our collaboration, and option and license agreements, as applicable. To the extent that we raise additional capital through the sale of equity or equity-linked securities, including convertible debt, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our existing stockholders' rights as holders of our common stock. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, obtaining additional capital, acquiring or divesting businesses, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances, or option and license arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

We enter into agreements in the normal course of business with clinical research organizations, contract manufacturing organizations, and institutions to license intellectual property. These contracts generally are cancelable at any time by us, upon 30 to 90 days prior written notice.

Our agreements to license intellectual property include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. We may also be required to pay annual maintenance fees or minimum amounts payable ranging from low-four digits to low five-digits depending upon the terms of the applicable agreement. In certain instances, we are also obligated to pay our licensors royalties based on sales of products, if approved, using the intellectual property licensed under the applicable agreement.

We also have non-cancelable operating lease commitments arising from our leases of office and laboratory space at our facilities in Cambridge and Lexington, Massachusetts. For more information, refer to Note 7 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable rules of the Securities and Exchange Commission, or the SEC.

Smaller Reporting Company Status

As of June 30, 2022, we have requalified as a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to qualify as a smaller reporting company if we have (a) a non-affiliate public float in excess of $250 million and annual revenues in excess of $100 million during our last fiscal year, or (b) a non-affiliate public float in excess of $700 million, in each case determined on an annual basis as of the last business day of our second quarter. As a smaller reporting company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies. These exemptions include:

- being permitted to provide only two years of audited consolidated financial statements in this Annual Report on Form 10-K, with correspondingly reduced Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- reduced disclosure obligations regarding executive compensation; and
- not being required to furnish a stock performance graph in our annual report.

We expect to take advantage of some or all of the available exemptions until we cease to be a smaller reporting company. We may cease to qualify as a smaller reporting company as early as June 30, 2023, which would require us to comply with disclosure requirements that are applicable to other public companies that are not smaller reporting companies following the filing of our Annual Report on Form 10-K for the year ending December 31, 2023, and any portions of our definitive proxy statement relating to our 2024 Annual Meeting of Stockholders incorporated by reference therein.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to market risk related to changes in interest rates. We have policies requiring us to invest in high-quality issuers, limit our exposure to any individual issuer, and ensure adequate liquidity. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of money market funds and marketable securities and are invested in U.S. Treasury notes. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, we believe an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We are not currently exposed to market risk related to changes in foreign currency exchange rates; however, we may contract with vendors that are located in Asia and Europe in the future and may be subject to fluctuations in foreign currency rates at that time.

Inflation generally affects us by increasing our costs of labor, goods, and services. We do not believe that inflation had, or that an immediate 100 basis point change in inflation would have had, a material effect on our business, financial condition, or results of operations during the year ended December 31, 2022.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Management's Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(c) or 15d-15(e) under the Exchange Act to mean controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Our disclosure controls and procedures include, without limitation, controls and other procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2022. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have

concluded based upon the evaluation described above that, as of December 31, 2022, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act as a process designed by, or under the supervision of, a company's principal executive officer and principal financial officer, or persons performing similar functions, and effected by a company's board of directors, management, and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of a company's assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that a company's receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision of and with the participation of our principal executive officer and principal financial and accounting officer, our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2022 based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control—Integrated Framework (2013 framework). Based on this assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2022.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during our fiscal quarter ended December 31, 2022 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Not applicable.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Incorporated by reference from the information in our Proxy Statement for our 2023 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 11. EXECUTIVE COMPENSATION

Incorporated by reference from the information in our Proxy Statement for our 2023 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Incorporated by reference from the information in our Proxy Statement for our 2023 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Incorporated by reference from the information in our Proxy Statement for our 2023 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

Incorporated by reference from the information in our Proxy Statement for our 2023 Annual Meeting of Stockholders, which we expect to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)(1) Financial Statements.

	Pages
Report of independent registered public accounting firm PCAOB ID 42	F- 1
Consolidated Balance Sheets	F-3
Consolidated Statements of Operations and Comprehensive (Loss) Income	F-4
Consolidated Statements of Stockholders' Equity	F-5
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(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Consolidated Financial Statements or Notes thereto set forth under Item 8 above.

(a)(3) Exhibits.

See the Exhibit Index immediately preceding the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index below are filed or incorporated by reference as part of this Annual Report on Form 10-K.

ITEM 16. FORM 10-K SUMMARY

This Annual Report on Form 10-K does not include a summary.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Voyager Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Voyager Therapeutics, Inc. (the "Company") as of December 31, 2022 and 2021, the related consolidated statements of operations and comprehensive (loss) income, stockholders' equity, and cash flows for each of the three years in the period ended December 31, 2022, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2022 and 2021, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2022, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Revenue recognition under the proportional performance model

Description of the Matter	As discussed in Note 9 to the consolidated financial statements, in 2019 the Company entered into a Collaboration Agreement which resulted in collaboration revenue of $0.9 million for the year ended December 31, 2022 and deferred revenue of $11.8 million as of December 31, 2022. The Company recognizes consideration allocated to each performance obligation using the proportional performance method. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs.

	Auditing collaboration revenue recognized was especially challenging and judgmental because the proportional performance calculation involves subjective management assumptions about estimates of the expected remaining costs to complete the research and development services for each performance obligation. Changes in expected remaining costs to complete can have a material effect on the amount of collaboration revenue recognized.
How We Addressed the Matter in Our Audit	Our audit procedures included, among others, the inspection of the Company's contract and testing of the completeness and accuracy of the underlying data used to determine the expected remaining costs to complete the research and development services for each performance obligation. We performed inquiries of research and development personnel to validate management's estimates and obtained corroborative evidence to assess the reasonableness of the proportional performance calculation. We also performed a retrospective review to assess the Company's historical estimates of the remaining costs to complete the research and development services and a sensitivity analysis to evaluate the materiality of reasonable changes in management's assumptions.

/s/ *Ernst & Young LLP*

We have served as the Company's auditor since 2015.

Boston, Massachusetts
March 7, 2023

Voyager Therapeutics, Inc.
Consolidated Balance Sheets
(amounts in thousands, except share and per share data)

	December 31,	
	2022	**2021**
Assets		
Current assets:		
Cash and cash equivalents	$ 98,959	$ 117,433
Marketable securities	19,889	15,106
Related party collaboration receivable	257	732
Prepaid expenses and other current assets	5,394	3,427
Total current assets	124,499	136,698
Property and equipment, net	17,857	21,920
Deposits and other non-current assets	1,515	1,779
Operating lease, right-of-use assets	15,485	33,458
Total assets	$ 159,356	$ 193,855
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,566	$ 574
Accrued expenses	7,816	10,950
Other current liabilities	2,832	5,571
Deferred revenue, current	59,377	33,886
Total current liabilities	72,591	50,981
Deferred revenue, non-current	6,450	8,210
Other non-current liabilities	21,295	39,609
Total liabilities	100,336	98,800
Commitments and contingencies (see note 9)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized; no shares issued and outstanding at December 31, 2022 and 2021	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 38,613,891 and 37,918,395 shares issued and outstanding at December 31, 2022 and 2021, respectively	38	38
Additional paid-in capital	452,713	442,259
Accumulated other comprehensive loss	(219)	(138)
Accumulated deficit	(393,512)	(347,104)
Total stockholders' equity	59,020	95,055
Total liabilities and stockholders' equity	$ 159,356	$ 193,855

The accompanying notes are an integral part of these consolidated financial statements.

Voyager Therapeutics, Inc.
Consolidated Statements of Operations and Comprehensive (Loss) Income
(amounts in thousands, except share and per share data)

	Year ended December 31,		
	2022	**2021**	**2020**
Collaboration revenue	$ 40,907	$ 37,415	$ 171,128
Operating expenses:			
Research and development	60,764	73,787	108,753
General and administrative	30,980	37,246	34,991
Total operating expenses	91,744	111,033	143,744
Operating (loss) income	(50,837)	(73,618)	27,384
Other income, net:			
Interest income (expense)	1,792	(390)	1,659
Other income	2,653	2,811	7,698
Total other income, net	4,445	2,421	9,357
(Loss) income before income taxes	(46,392)	(71,197)	36,741
Income tax provision	16	—	—
Net (loss) income	$ (46,408)	$ (71,197)	$ 36,741
Other comprehensive (loss) income			
Net unrealized loss on available-for-sale-securities	(81)	(4)	(30)
Total other comprehensive loss	(81)	(4)	(30)
Comprehensive (loss) income	$ (46,489)	$ (71,201)	$ 36,711
Net (loss) income per share, basic	$ (1.21)	$ (1.89)	$ 0.99
Net (loss) income per share, diluted	(1.21)	(1.89)	$ 0.98
Weighted-average common shares outstanding, basic	38,356,810	37,668,947	37,132,447
Weighted-average common shares outstanding, diluted	38,356,810	37,668,947	37,348,514

The accompanying notes are an integral part of these consolidated financial statements.

Voyager Therapeutics, Inc.
Consolidated Statements of Stockholders' Equity
(amounts in thousands, except share data)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity
Balance at December 31, 2019	36,865,116	$ 37	$ 412,227	$ (104)	$ (312,648)	$ 99,512
Exercises of vested stock options	228,436	—	2,319	—	—	2,319
Vesting of restricted stock units	170,367	—	—	—	—	—
Issuance of common stock under ESPP	104,108	—	1,279	—	—	1,279
Stock-based compensation expense	—	—	14,499	—	—	14,499
Unrealized loss on available-for-sale securities	—	—	—	(30)	—	(30)
Net income	—	—	—	—	36,741	36,741
Balance at December 31, 2020	37,368,027	$ 37	$ 430,324	$ (134)	$ (275,907)	$ 154,320
Exercises of vested stock options	3,811	1	27	—	—	28
Vesting of restricted stock units	346,551	—	—	—	—	—
Issuance of common stock under ESPP	200,006	—	918	—	—	918
Stock-based compensation expense	—	—	10,990	—	—	10,990
Unrealized loss on available-for-sale securities	—	—	—	(4)	—	(4)
Net loss	—	—	—	—	(71,197)	(71,197)
Balance at December 31, 2021	37,918,395	$ 38	$ 442,259	$ (138)	$ (347,104)	$ 95,055
Exercises of vested stock options	89,012	—	629	—	—	629
Vesting of restricted stock units	456,219	—	—	—	—	—
Issuance of common stock under ESPP	150,265	—	672	—	—	672
Stock-based compensation expense	—	—	9,153	—	—	9,153
Unrealized loss on available-for-sale securities	—	—	—	(81)	—	(81)
Net loss	—	—	—	—	(46,408)	(46,408)
Balance at December 31, 2022	38,613,891	$ 38	$ 452,713	$ (219)	$ (393,512)	$ 59,020

The accompanying notes are an integral part of these consolidated financial statements

Voyager Therapeutics, Inc.
Consolidated Statements of Cash Flows
(amounts in thousands)

	Year ended December 31,		
	2022	**2021**	**2020**
Cash flow from operating activities			
Net (loss) income	$ (46,408)	$ (71,197)	$ 36,741
Adjustments to reconcile net (loss) income to net cash used in operating activities:			
Stock-based compensation expense	9,344	11,324	14,934
Depreciation	6,191	5,165	3,817
Amortization of premiums and discounts on marketable securities	(16)	349	27
Gain on Lease Termination	(2,468)	—	—
Change in fair value of common stock and warrants to purchase equity securities	—	(2,460)	(7,698)
Loss on disposal of fixed assets	377	—	—
Changes in operating assets and liabilities:			
Related party collaboration receivable	475	7,280	10,484
Prepaid expenses and other assets	(1,967)	1,883	(551)
Operating lease, right-of-use assets	3,462	2,606	(7,592)
Other non-current assets	(152)	69	275
Accounts payable	1,992	(60)	(3,436)
Accrued expenses	(3,148)	(3,335)	(6,480)
Operating lease liabilities	(3,922)	(3,428)	13,439
Deferred revenue	23,731	(1,721)	(150,676)
Net cash used in operating activities	(12,509)	(53,525)	(96,716)
Cash flow from investing activities			
Purchases of property and equipment	(2,491)	(1,609)	(12,097)
Purchases of marketable securities	(54,848)	(15,117)	(70,403)
Proceeds from sales and maturities of marketable securities	50,000	82,632	195,495
Net cash (used in) provided by investing activities	(7,339)	65,906	112,995
Cash flow from financing activities			
Proceeds from the exercise of stock options	629	28	2,319
Proceeds from the purchase of common stock under ESPP	481	584	844
Net cash provided by financing activities	1,110	612	3,163
Net (decrease) increase in cash and cash equivalents	(18,738)	12,993	19,442
Cash, cash equivalents, and restricted cash beginning of period	119,212	106,219	86,777
Cash, cash equivalents, and restricted cash end of period	$ 100,474	$ 119,212	$ 106,219
Supplemental disclosure of cash and non-cash activities			
Operating lease right-of-use assets obtained in exchange for operating lease liabilities	$ —	$ 664	$ 10,818
Capital expenditures incurred but not yet paid	$ 14	$ 80	$ 831

The accompanying notes are an integral part of these consolidated financial statements.

VOYAGER THERAPEUTICS INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of business

Voyager Therapeutics, Inc. (the "Company") is a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. The Company focuses on leveraging its expertise in capsid discovery and neuropharmacology to address the delivery hurdles that have constrained the gene therapy and neurology disciplines, with the goal of either halting or slowing disease progression or reduce symptom severity, therefore providing clinically meaningful impact to patients. The Company's gene therapy platforms enable it to engineer, optimize, manufacture and deliver its adeno-associated virus ("AAV") based gene therapies that it believes have the potential to safely provide durable efficacy. The Company's team of experts in the field of AAV gene therapy and neuroscience first identifies and selects diseases in which the Company believes an AAV gene therapy or other biological therapy will answer a high unmet medical need, be supported by target validation, offer an efficient path to human proof of biology, present robust preclinical pharmacology, and offer strong commercial potential. The Company then engineers and optimizes an AAV vector or other biological therapy for activity in, efficacy in, or delivery to, the targeted tissue or cells.

The Company is identifying proprietary AAV capsids, the outer viral protein shells that enclose genetic material that makes up the vector payload. The Company's team has developed a proprietary AAV capsid discovery platform called TRACER™ (Tropism Redirection of AAV by Cell Type-Specific Expression of RNA) that employs directed evolution to facilitate the selection of AAV capsids with enhanced tissue delivery characteristics, such as more effective delivery across the blood brain barrier ("BBB"). The TRACER discovery platform is a broadly applicable, functional RNA-based AAV capsid discovery platform that allows for rapid *in vivo* evolution of AAV capsids with cell-specific transduction properties in multiple species, including non-human primates. The Company believes that the capsids it discovers through its TRACER discovery platform ("TRACER Capsids") have the potential to significantly enhance the efficacy and safety of its single dose gene therapies, which it expects to be delivered with targeted surgical delivery or systemic infusions, as compared with conventional capsids.

The Company has a history of incurring annual net operating losses. As of December 31, 2022, the Company had an accumulated deficit of $393.5 million. The Company has not generated any product revenue and has financed its operations primarily through public offerings and private placements of its equity securities and funding from fees, milestone payments, and cost reimbursements associated with its prior collaborations with Sanofi Genzyme Corporation ("Sanofi Genzyme") and AbbVie Biotechnology Ltd and AbbVie Ireland Unlimited Company (collectively, "AbbVie"), and its ongoing collaborations with Neurocrine Biosciences, Inc. ("Neurocrine"), its option and license agreement with Pfizer, Inc. ("Pfizer"), and its option and license agreement with Novartis Pharma AG ("Novartis").

As of December 31, 2022, the Company had cash, cash equivalents, and marketable securities of $118.8 million. Based upon its current operating plan, the Company expects that its existing cash, cash equivalents, and marketable securities at December 31, 2022, together with the upfront payment received in February 2023 in connection with the Collaboration and License Agreement by and between the Company and Neurocrine dated as of January 8, 2023 (the "2023 Neurocrine Collaboration Agreement"), along with amounts expected to be received as reimbursement for development costs under the Company's collaboration and license agreements with Neurocrine, will be sufficient to meet the Company's planned operating expenses and capital expenditure requirements into 2025.

There can be no assurance that the Company will be able to obtain additional debt or equity financing on terms acceptable to the Company or generate product revenue or revenue from collaboration partners, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of significant accounting policies and basis of presentation

Basis of presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and pursuant to the rules and regulations of the U.S. Securities and Exchange Commission ("SEC") for reporting on Form 10-K. The Company's consolidated financial statements include the accounts of Voyager Therapeutics, Inc. and its wholly-owned subsidiary, Voyager Securities Corporation. All intercompany balances and transactions have been eliminated.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, accrued expenses, stock-based compensation expense, and income taxes. The Company bases its estimates on historical experience and other market specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

Fair Value of Financial Instruments

ASC Topic 820, *Fair Value Measurement* ("ASC 820"), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances.

ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- *Level 1*—Quoted market prices in active markets for identical assets or liabilities.
- *Level 2*—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates, and yield curves.
- *Level 3*—Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The carrying amounts reflected in the balance sheets for cash and cash equivalents, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values, due to their short-term nature.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.

Marketable Securities

The Company classifies marketable securities with a remaining maturity of greater than three months when purchased as available-for-sale. Marketable securities with a remaining maturity date greater than one year and marketable equity securities are classified as non-current where the Company has the intent and ability to hold these securities for at least the next 12 months.

All available for sale debt securities are carried at fair value with the unrealized gains and losses included in other comprehensive (loss) income as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense. Realized gains and losses are determined using the specific identification method and are included in other income. If any adjustment to fair value reflects a decline in value of the investment, the Company uses a forward-looking approach based on expected losses to estimate credit losses on certain types of financial instruments, including trade receivables and available-for-sale debt securities. No other than temporary losses have been recognized.

Cash, cash equivalents, and marketable securities as of December 31, 2022 and 2021 consist of the following:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
		(in thousands)		
As of December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	—	—	$ 91,724
Marketable securities- U.S. Treasury notes	19,980	—	(91)	19,889
Total money market funds and marketable securities	$ 111,704	$ —	$ (91)	$ 111,613
As of December 31, 2021				
Money market funds included in cash and cash equivalents	$ 100,305	$ —	$ —	$ 100,305
Marketable securities- U.S. Treasury notes	15,117	—	(11)	15,106
Total money market funds and marketable securities	$ 115,422	$ —	$ (11)	$ 115,411

All of the Company's marketable securities at December 31, 2022 and 2021 have a contractual maturity of one year or less.

Restricted Cash

As of December 31, 2022 and 2021, the Company maintained restricted cash totaling approximately $1.5 million and $1.8 million, respectively, held in the form of money market accounts as collateral for the Company's facility lease obligations. The balance is included within deposits and other non-current assets in the accompanying consolidated balance sheets. The following table provides a reconciliation of cash, cash equivalents, and restricted cash within the consolidated balance sheets that sum to the total of the same such amounts shown in the statements of cash flows:

	As of December 31,		
	2022	2021	2020
		(in thousands)	
Cash and cash equivalents	$ 98,959	$ 117,433	$ 104,440
Restricted cash included in deposits and other non-current assets	1,515	1,779	1,779
Total cash, cash equivalents, and restricted cash	$ 100,474	$ 119,212	$ 106,219

Property and Equipment

Property and equipment consists of laboratory equipment, furniture and office equipment, and leasehold improvements and is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred; while costs of major additions and

betterments are capitalized. Depreciation is calculated over the estimated useful lives of the assets using the straight-line method.

Impairment of Long-Lived Assets

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book values of the assets exceed their fair value. The Company has not recognized any impairment losses from inception through December 31, 2022.

Revenue Recognition

The Company enters into license, option, and collaboration agreements which are within the scope of ASC 606, *Revenue from Contracts with Customers* ("ASC 606"), under which the Company licenses or provides options to license certain of the Company's product candidates and, in certain cases, performs research and development services. The terms of these arrangements typically include payment of one or more of the following: non-refundable, upfront fees; reimbursement of research and development costs; option exercise fees; development, regulatory, and commercial milestone payments; and royalties on net sales of licensed products.

The promised goods or services in the Company's arrangements typically consist of license rights to the Company's intellectual property and research and development services. The Company provides options to additional items in the contracts, which are accounted for as separate contracts when the customer elects to exercise such options, unless the option provides a material right to the customer. The Company evaluates the customer options for material rights, or options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer and are considered distinct when (a) the customer can benefit from the good or service on its own or together with other readily available resources and (b) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on its own or whether the required expertise is readily available and whether the goods or services are integral or dependent to other goods or services in the contract.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of potential payments and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected amount method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration which is included in the transaction price may be constrained, and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

The Company's contracts often include development and regulatory milestone payments which are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within the Company's control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenue in the period of adjustment. To date, the Company has not recognized any consideration related to the achievement of development, regulatory, or commercial milestone revenue resulting from any of the Company's collaboration or license arrangements.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (a) when the related sales occur, or (b) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any consideration related to sales-based royalty revenue resulting from any of the Company's collaboration or license arrangements.

The Company allocates the transaction price based on the estimated stand-alone selling price of each of the performance obligations. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes key assumptions to determine the stand-alone selling price for service obligations, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Additionally, in determining the standalone selling price for material rights, the Company utilizes comparable transactions, clinical trial success probabilities, and estimates of option exercise likelihood. Variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts the Company would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Upfront payments and fees are recorded as contract liabilities within deferred revenue on the consolidated balance sheets until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. A significant portion of revenue recognized from the 2019 Neurocrine Collaboration Agreement is related to performance obligations pursuant to which revenue is recognized using a proportional performance model. Revenue is recognized using input-based measurements, which involves the measurement of progress toward each performance obligation based on the actual costs incurred compared to total projected costs. The Company estimates the expected remaining costs to complete the research and development services for each performance obligation. The Company evaluates the measure of progress each reporting period and, if necessary, adjust the measure and related revenue recognition.

Research and Development

Research and development costs are charged to expense as incurred in performing research and development activities. The costs include employee compensation costs, external research, consultant costs, sponsored research, license fees, process development and facilities costs. Facilities costs primarily include the allocation of rent, utilities and depreciation.

Leases

The Company determines if an arrangement is or contains a lease at inception under Accounting Standards Codification (ASC) 842 *Leases*. For leases with a term of 12 months or less, the Company does not recognize a right-of-use asset or lease liability. The Company's operating leases are recognized on its consolidated balance sheet as operating lease, right-of-use asset, other current liabilities, and other non-current liabilities. The Company does not have any finance leases.

Right-of-use assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease right-of-use assets and liabilities are recognized at the lease commencement date based on the present value of lease payments over the lease term. As the Company's leases typically do not provide an implicit rate, the Company uses an estimate of its incremental borrowing rate based on the information available at the lease commencement date in determining the

present value of lease payments. Operating lease right-of-use assets also include the effect of any lease prepaid or deferred lease payments and are reduced by lease incentives. The lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Lease expense is recognized on a straight-line basis over the lease term.

The Company has lease agreements with lease and non-lease components, which are generally accounted for separately. Non-lease components as it pertains to the Company's leased premises generally refer to common area maintenance charges related to the premises.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

The Company expenses patent application and related legal costs as incurred and classifies such costs as general and administrative expenses in the accompanying statements of operations.

Stock-Based Compensation Expense

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718 *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, directors, and other service providers, referred to as non-employees, including grants of restricted stock units and stock options, to be recognized as expense in the consolidated statements of operations based on their grant date fair values. The Company estimates the fair value of options granted using the Black-Scholes option pricing model. The Company uses the fair value of its common stock to determine the fair value of restricted stock awards and restricted stock units.

The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (a) the expected stock price volatility, (b) the expected term of the award, (c) the risk-free interest rate and (d) expected dividends. The Company bases the estimate of expected volatility on the historical volatility of its common stock. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The Company uses the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for stock options granted to employees as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. For stock options granted to non-employees, the Company utilizes the contractual term of the arrangement as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock.

The Company expenses the fair value of its stock-based compensation awards on a straight-line basis over the associated service period, which is generally the period in which the related services are received, adjusted for actual forfeitures of unvested awards as they occur.

The Company records the expense for stock-based compensation awards subject to performance conditions over the remaining service period when management determines that achievement of the performance condition is probable. Management evaluates when the achievement of a performance condition is probable based on the expected satisfaction of the performance conditions as of the reporting date.

Income Taxes

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial reporting and the tax reporting basis of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse. The Company provides a valuation allowance against net deferred tax assets unless, based upon the weight of available evidence, it is more likely than not that the deferred tax assets will be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2022, the Company does not have any significant uncertain tax positions.

Comprehensive (Loss) Income

Comprehensive (loss) income is comprised of net (loss) income and other comprehensive income or loss. Other comprehensive income or loss consists of unrealized gains or losses on marketable securities.

Net (Loss) Income Per Share

Basic net (loss) income per share is calculated by dividing the net (loss) income by the weighted-average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Diluted net (loss) income per share is computed by dividing the net (loss) income by the weighted-average number of shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury-stock and if-converted methods.

For purposes of the diluted net (loss) income per share, unvested restricted common stock and outstanding stock options are considered to be potentially dilutive securities. Unvested restricted common stock and outstanding stock options were excluded from the calculation of diluted net loss per share in the years ended December 31, 2022 and 2021, because their effect would be anti-dilutive and therefore, basic and diluted net loss per share were the same for the years ended December 31, 2022 and 2021.

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net (loss) income per share because to do so would be anti-dilutive:

	As of December 31,		
	2022	2021	2020
Unvested restricted common stock awards	45,000	137,255	156,863
Unvested restricted common stock units	1,112,563	806,379	527,625
Outstanding stock options	6,199,571	5,013,193	5,379,856
Total	7,357,134	5,956,827	6,064,344

Basic net (loss) income and diluted weighted-average shares outstanding are as follows for the years ended December 31, 2022, 2021, and 2020:

	Year Ended December 31,		
	2022	2021	2020
	(in thousands, except share data)		
Numerator:			
Net (loss) income	$ (46,408)	$ (71,197)	$ 36,741
Denominator for basic net (loss) income per share:			
Weighted average shares outstanding-basic	38,356,810	37,668,947	37,132,447
Denominator for diluted net (loss) income per share:			
Weighted average shares outstanding	38,356,810	37,668,947	37,132,447
Common stock options and restricted stock units	—	—	216,068
Weighted average shares outstanding-diluted	38,356,810	37,668,947	37,348,514

Concentrations of Credit Risk and Significant Suppliers

The Company has no financial instruments with off-balance sheet risk such as foreign exchange contracts, option contracts or other foreign currency hedging arrangements. Financial instruments that potentially subject the Company to a concentration of credit risk are cash and cash equivalents. The Company's cash is held in accounts at financial institutions that may exceed federally insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds.

The Company is dependent on third-party manufacturers to supply certain products for research and development activities in its programs. In particular, the Company relies on a sole manufacturer to supply it with specific vectors related to the Company's research and development programs.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker in deciding how to allocate resources and assess performance. The Company and the Company's chief operating decision maker, the Company's Chief Executive Officer, view the Company's operations and manages its business as a single operating segment, which is the business of developing and commercializing gene therapies.

Recent Accounting Pronouncements

In August 2020, the FASB issued ASU No. 2020-06, *Debt – Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40)*. This standard amends the guidance on convertible instruments and the derivatives scope exception for contracts in an entity's own equity and amends the related earnings per share ("EPS") guidance. The ASU will be effective for smaller reporting companies for fiscal years beginning after December 15, 2023 and interim periods within those fiscal years. Early adoption is permitted in fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The Company is assessing the impact of ASU 2020-06 on the consolidated financial statements and does not expect it to have a material impact.

3. Fair value measurements

Assets and liabilities measured at fair value on a recurring basis as of December 31, 2022 and 2021 are as follows:

Assets	Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
		(in thousands)		
December 31, 2022				
Money market funds included in cash and cash equivalents	$ 91,724	$ 91,724	$ —	$ —
Marketable securities- U.S. Treasury notes	19,889	19,889	—	—
Total	$ 111,613	$ 111,613	$ —	$ —
December 31, 2021				
Money market funds included in cash and cash equivalents	$ 100,305	$ 100,305	$ —	$ —
Marketable securities- U.S. Treasury notes	15,106	15,106		
Total	$ 115,411	$ 115,411	$ —	$ —

The Company measures the fair value of money market funds and U.S. Treasuries based on quoted prices in active markets for identical securities.

4. Prepaid expenses and other current assets

Prepaid expenses and other current assets consist of the following:

	As of December 31, 2022	As of December 31, 2021
	(in thousands)	
Other current assets	$ 4,233	$ 1,701
Prepaid insurance	696	1,360
Prepaid research and development contracts	83	350
Accrued interest receivable	382	16
Total	$ 5,394	$ 3,427

5. Property and equipment, net

Property and equipment, net consists of the following:

	As of December 31, 2022	As of December 31, 2021
	(in thousands)	
Laboratory equipment	$ 19,675	$ 19,384
Leasehold improvements	12,554	15,695
Furniture and office equipment	2,333	2,524
Other	502	230
Total property and equipment	35,064	37,833
Less: accumulated depreciation	(17,207)	(15,913)
Property and equipment, net	$ 17,857	$ 21,920

The Company recorded $6.2 million, $5.2 million, and $3.8 million in depreciation expense during the years ended December 31, 2022, 2021, and 2020, respectively.

6. Accrued expenses

Accrued expenses consist of the following:

	As of December 31,	
	2022	2021
	(in thousands)	
Employee compensation costs	$ 4,559	$ 5,022
Research and development costs	1,895	3,719
Accrued goods and services	636	1,482
Professional services	726	727
Total	$ 7,816	$ 10,950

7. Lease obligation

Operating Leases

As of December 31, 2022, the Company has a lease for office and laboratory space at 64 Sidney Street in Cambridge, Massachusetts through November 30, 2026 and a lease for additional laboratory and office space at 75 Hayden Avenue in Lexington, Massachusetts through January 31, 2031.

In September 2021, the Company entered into an agreement with BioNTech US, Inc. ("BioNTech US") to sublease part of the office and laboratory space leased by the Company at 75 Sidney Street in Cambridge, Massachusetts (the "Sublease Agreement") at that time. The sublease term was for approximately 3.3 years. The sublease did not relieve the Company of its original obligation under the lease, and therefore the Company did not adjust the operating lease right-of-use asset as a result of the sublease and accounted for the sublease as a separate lease.

On June 22, 2022 the Company entered into a Lease Termination Agreement (the "Lease Termination Agreement") and terminated the lease for office and laboratory space at 75 Sidney Street (the "75 Sidney Street Lease"), effective immediately. In connection with the Lease Termination Agreement, the Company also entered into a Sublease Termination Agreement (the "Sublease Termination Agreement") and terminated the Sublease Agreement with BioNTech US. The Company did not incur any termination penalties in connection with the Lease Termination Agreement or Sublease Termination Agreement. The Company derecognized the related right-of-use asset of approximately $14.5 million and the operating lease liabilities of $17.0 million, accordingly, resulting in a gain of $2.5 million in the three-month period ended June 30, 2022.

The Company's lease agreements require the Company to maintain a cash deposit or irrevocable letter of credit in the aggregate amount of $1.5 million payable to its landlords as security for the performance of its obligations under the leases. These amounts are recorded as restricted cash and are included in deposits and other non-current assets in the accompanying consolidated balance sheets.

Total lease cost for operating leases of approximately $4.6 million, $6.8 million, and $6.2 million was incurred during the years ended December 31, 2022, 2021 and 2020, respectively. As of December 31, 2022, the weighted average remaining lease term was 6 years and the weighted average incremental borrowing rate used to determine the operating lease liabilities was 7.4%.

The following table summarizes the operating sublease income generated under the Sublease Agreement which was recorded within operating expenses for the years ended December 31, 2022 and 2021.

	Years ended December 31,	
	2022	2021
	(in thousands)	
Operating sublease income	$ 1,380	$ 838

8. Other liabilities

As of December 31, 2022 and 2021, other current and non-current liabilities consisted of the following:

	As of December 31,	
	2022	2021
	(in thousands)	
Other current liabilities		
Lease liabilities	2,832	5,571
Total other current liabilities	$ 2,832	$ 5,571
Other non-current liabilities		
Lease liabilities	$ 20,294	$ 38,608
Other	1,001	1,001
Total other non-current liabilities	$ 21,295	$ 39,609

Strategic Restructuring

On August 6, 2021, the board of directors of the Company approved a strategic restructuring plan to eliminate a portion of its workforce as part of an initiative to reduce expenses and enhance operations. The strategic restructuring plan was approved in connection with its portfolio reevaluation efforts and its strategic shift to invest additional resources in the Company's TRACER capsid development efforts.

During the year ended December 31, 2021, the Company incurred restructuring costs of approximately $2.6 million, which consists of severance-related costs. These costs are reported within our research and development expenses and general and administrative expenses. Substantially all costs have been paid as of December 31, 2022.

9. Commitments and contingencies

Significant Agreements

2019 Neurocrine Collaboration Agreement

Summary of Agreement

Effective March 2019, the Company entered into a collaboration agreement with Neurocrine (the "2019 Neurocrine Collaboration Agreement") for the research, development and commercialization of certain of its AAV gene therapy products. Under the 2019 Neurocrine Collaboration Agreement, the Company agreed to collaborate on the conduct of four collaboration programs (the "2019 Neurocrine Programs") which include: (a) VY-AADC (NBIb-1817) for Parkinson's disease (the "VY-AADC Program"), (b) VY-FXN01 for Friedreich's ataxia (the "FA Program") (collectively, with the VY-AADC Program, the "Legacy Programs"), and (c) two programs to be determined by the Company and Neurocrine at a later date (the "2019 Discovery Programs").

In June 2019, in conjunction with the termination of the collaboration agreement with Sanofi Genzyme (the "Sanofi Genzyme Collaboration Agreement"), the Company gained ex-U.S. rights to the FA Program. The Company's ex-U.S. rights to the FA Program were subsequently transferred to Neurocrine under the terms of the 2019 Neurocrine Collaboration Agreement. To facilitate the transfer of the ex-U.S. rights to the FA Program to Neurocrine, the Company and Neurocrine executed an amendment to the 2019 Neurocrine Collaboration Agreement (the "June 2019 Modification"), and Neurocrine paid $5.0 million to the Company. There were no other changes in pricing or scope of the obligations required to be performed under the 2019 Neurocrine Collaboration Agreement.

In February 2021, Neurocrine notified the Company that it had elected to terminate the 2019 Neurocrine Collaboration Agreement solely with regards to the VY-AADC Program, effective August 2, 2021 (the "Neurocrine VY-AADC Program Termination Effective Date"). The 2019 Neurocrine Collaboration Agreement remains in full force and

effect for each other program thereunder. As a result of the termination, Neurocrine is no longer obligated to reimburse the Company for research and development activities related to the VY-AADC Program.

Under the terms of the 2019 Neurocrine Collaboration Agreement, the Company originally agreed to collaborate with Neurocrine on, and to grant, exclusive, royalty-bearing, non-transferable, sublicensable licenses to certain of its intellectual property rights, for all human and veterinary diagnostic, prophylactic, and therapeutic uses, for the research, development, and commercialization of gene therapy products (the " 2019 Collaboration Products") under (a) the VY-AADC Program on a worldwide basis; (b) the FA Program in the United States and, all countries in the world in which the 2019 Neurocrine Collaboration Agreement remains in effect with respect to the FA Program; and (c) each 2019 Discovery Program on a worldwide basis. As a result of the termination of the 2019 Neurocrine Collaboration Agreement with regards to the VY-AADC Program, in accordance with the terms of the 2019 Neurocrine Collaboration Agreement, the licenses granted by the Company to Neurocrine regarding the VY-AADC Program have expired, and the Company has regained worldwide intellectual property rights regarding the VY-AADC Program, in each case as of the VY-AADC Termination Effective Date.

Pursuant to development plans agreed by the parties, which are overseen by a joint steering committee ("JSC"), the Company has operational responsibility, subject to certain exceptions, for the conduct of each 2019 Neurocrine Program prior to the occurrence of a specified event for such 2019 Neurocrine Program (a "2019 Transition Event"), as described below, and is required to use commercially reasonable efforts to develop the corresponding 2019 Collaboration Products. Neurocrine has agreed to be responsible for all costs incurred by the Company in conducting these activities for each 2019 Neurocrine Program, in accordance with an agreed budget for each 2019 Neurocrine Program. If the Company breaches its development responsibilities or in certain circumstances upon a change in control, Neurocrine has the right but not the obligation to assume the activities under such 2019 Neurocrine Program.

Upon the occurrence of a 2019 Transition Event for each 2019 Neurocrine Program, Neurocrine has agreed to assume responsibility for development, manufacturing and commercialization activities for such 2019 Neurocrine Program from the Company and to pay milestones and royalties on future net sales as described further below. As a result of Neurocrine's termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the 2019 Transition Event with respect to the VY-AADC Program is no longer applicable. The 2019 Transition Events for the remaining programs are (a) with respect to the FA Program, the Company's receipt of topline data for the initial Phase 1 clinical trial for an FA Program product candidate; and (b) with respect to each 2019 Discovery Program, the preparation by the Company and the approval by Neurocrine of an IND application to be filed with the U.S. Food and Drug Administration (the "FDA") by Neurocrine for the first development candidate in such 2019 Discovery Program. For the FA Program, the Company was granted the option (the "2019 FA Co-Co Option") to co-develop and co-commercialize the FA Program upon the occurrence of a specified event (a "2019 FA Co-Co Trigger Event"). The Company agreed, upon its exercise of the FA Co-Co Option, to enter into a cost- and profit-sharing arrangement with Neurocrine (the "2019 FA Co-Co Agreement"), and (a) jointly develop and commercialize the 2019 Collaboration Products for the FA Program ("FA Collaboration Products"), (b) share in its costs, profits and losses, and (c) forfeit certain milestones and royalties on net sales in the United States during the effective period of the 2019 FA Co-Co Agreement. The 2019 FA Co-Co Trigger Event is the receipt of topline data for the initial Phase 1 clinical trial for a FA Program product candidate.

Under the 2019 Neurocrine Collaboration Agreement, subject to exceptions specified therein, the Company and Neurocrine agreed that profits and losses under the Company's 2019 FA Co-Co Option would be allocated 60% to Neurocrine and 40% to the Company for any FA Collaboration Product. The parties agreed that 2019 FA Co-Co Agreement would provide the Company the right to terminate for any reason upon prior written notice to Neurocrine and Neurocrine the right to terminate in certain circumstances upon change of control.

The Company's research and development activities under the 2019 Neurocrine Collaboration Agreement are conducted pursuant to plans agreed to by the parties, on a program-by-program basis, and overseen by the JSC, as detailed in the 2019 Neurocrine Collaboration Agreement.

Under the 2019 Neurocrine Collaboration Agreement, the parties committed to agree on a list of up to eight target genes (the "Targets") from which Neurocrine had the right to nominate Targets for the two 2019 Discovery

Programs. The Company and Neurocrine completed the nomination process, and the JSC has approved the two Targets for development under the 2019 Discovery Programs. The two Targets are currently under development.

The 2019 Neurocrine Collaboration Agreement provides for an upfront non-refundable payment of $115.0 million, as well as for aggregate development and regulatory milestone payments from Neurocrine to the Company for 2019 Collaboration Products under (a) the VY-AADC Program of up to $170.0 million, which the Company is no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) the FA Program of up to $195.0 million, and (c) each of the two 2019 Discovery Programs of up to $130.0 million per 2019 Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for each 2019 Collaboration Product of up to $275.0 million, subject to an aggregate cap on commercial milestone payments across all 2019 Neurocrine Programs of $1.1 billion. Furthermore, in connection with the 2019 Neurocrine Collaboration Agreement, Neurocrine purchased 4,179,728 shares of the Company's common stock at a price of $11.9625 per share, for an aggregate purchase price of $50.0 million.

Neurocrine also agreed to pay the Company royalties, based on future net sales of the 2019 Collaboration Products. Such royalty percentages, for net sales in and outside the United States, as applicable, range (a) for the VY-AADC Program, from the mid-teens to low thirties and the low-teens to low twenties, respectively, which the Company is no longer eligible to receive in light of the partial termination of the 2019 Neurocrine Collaboration Agreement; (b) for the FA Program, from the low-teens to high-teens and high-single digits to mid-teens, respectively; and (c) for each 2019 Discovery Program, from the high-single digits to mid-teens and mid-single digits to low-teens, respectively. On a country-by-country and program-by-program basis, royalty payments would commence on the first commercial sale of a 2019 Collaboration Product and terminate on the later of (a) the expiration of the last patent covering the 2019 Collaboration Product or its method of use in such country, (b) ten years from the first commercial sale of the 2019 Collaboration Product in such country and (c) the expiration of regulatory exclusivity in such country (the "2019 Royalty Term"). Royalty payments may be reduced by up to 50% in specified circumstances, including expiration of patents rights related to a 2019 Collaboration Product, approval of biosimilar products in a given country or required payment of licensing fees to third parties related to the development and commercialization of any 2019 Collaboration Product. As a result of Neurocrine's termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program, the Company is no longer entitled to receive royalties related to the VY-AADC Program. Additionally, the licenses granted to Neurocrine shall automatically convert to fully paid-up, non-royalty bearing, perpetual, irrevocable, exclusive licenses on a country-by-country and product-by-product basis upon the expiration of the 2019 Royalty Term applicable to such 2019 Collaboration Product in such country.

Under the terms of the 2019 Neurocrine Collaboration Agreement and subject to specified exceptions therein, each party owns the entire right, title and interest in and to all intellectual property rights made solely by its employees or agents in the course of the collaboration. The parties jointly own all rights, title and interest in and to all intellectual property rights made or invented jointly by employees or agents of both parties.

During the term of the 2019 Neurocrine Collaboration Agreement, neither party nor any of its respective affiliates is permitted to directly or indirectly exploit any AAV-based gene therapy products directed to a Target to which a 2019 Collaboration Product is directed, subject to specified exceptions, including the parties' conduct of basic research activities.

Unless earlier terminated, the 2019 Neurocrine Collaboration Agreement expires on the later of (a) the expiration of the last to expire 2019 Royalty Term with respect to a 2019 Collaboration Product in all countries in the relevant territory or (b) the expiration or termination of any 2019 FA Co-Co Agreement. Neurocrine may terminate the 2019 Neurocrine Collaboration Agreement in its entirety or on a program-by-program or country-by-country basis by providing at least (x) 180-day advance notice if such notice is provided prior to the first commercial sale of the 2019 Collaboration Product to which the termination applies or (y) one-year advance notice if such notice is provided after the first commercial sale of the 2019 Collaboration Product to which the termination applies. The Company may terminate the 2019 Neurocrine Collaboration Agreement, subject to specified conditions, if Neurocrine challenges the validity or enforceability of certain of the Company's intellectual property rights. Subject to a cure period, either party may terminate the 2019 Neurocrine Collaboration Agreement in the event of a material breach by the other party in whole or in part, subject to specified conditions.

Upon termination in certain cases, Neurocrine has agreed to grant to the Company licenses to certain Neurocrine intellectual property, subject to a negotiation between the parties to establish royalty rates for use of such intellectual property. In the event of a breach by the Company with respect to a 2019 Neurocrine Program, if such termination were to occur after a 2019 Transition Event, then (a) with respect to the FA Program, if a 2019 FA Co-Co Agreement is in effect, Neurocrine can terminate the 2019 FA Co-Co Agreement for such program and the Company would no longer have co-development and co-commercialization rights with respect to the FA Collaboration Products and (b) subject to any license agreements, Neurocrine would no longer have any obligations with respect to any 2019 Collaboration Products resulting from such program.

Termination of VY-AADC Program

As described above, as of the Neurocrine VY-AADC Program Termination Effective Date, the license granted by the Company to Neurocrine thereunder regarding the VY-AADC Program expired, the Company regained worldwide intellectual property rights regarding the VY-AADC Program, and the restrictions on the Company to develop, manufacture or commercialize a gene therapy product directed to the target of the VY-AADC Program terminated, in each case in accordance with the terms of the 2019 Neurocrine Collaboration Agreement. As of the Neurocrine VY-AADC Program Termination Effective Date, Neurocrine no longer is obligated to reimburse the Company for research and development activities related to the VY-AADC Program, and the Company is no longer entitled to receive future milestone or royalty payments related to the VY-AADC Program. The Company is supporting Neurocrine, the study sponsor and IND holder, on ongoing matters related to the completion of imaging and clinical assessments requested by the Data Safety and Monitoring Board, and the provision of other information requested by the FDA for the RESTORE-1 Phase 2 clinical trial.

Accounting Analysis

At inception, the Company determined the 2019 Neurocrine Collaboration Agreement was a contract with a customer under ASC 606, and included the following performance obligations: (a) research and development services for each Legacy Program combined with a development and commercialization license for each such program and (b) research and development services for each 2019 Discovery Program combined with a development and commercialization license for each program. The research services and license on a program-by-program basis are not distinct as Neurocrine cannot benefit from such license on its own or from other resources commonly available in the industry, without the corresponding research services due to the unique and specialized expertise of the Company that is not readily available in the marketplace.

The Company identified $92.4 million of fixed transaction price consisting of the $115.0 million upfront fee and $5.0 million payment from the June 2019 Modification, offset by a discount of $27.6 million related to the $50.0 million equity investment of 4,179,728 shares when measured at fair value on the date of issuance. The Company is also entitled to reimbursement of costs incurred by the Company prior to the 2019 Transition Events associated with each 2019 Neurocrine Program. These amounts are determinable based on program plans and budgets, and the Company has a contractual right to the payment of cost incurred under the agreed upon program plans. The Company utilized the most likely amount approach and estimated the expected cost reimbursement to be $431.1 million at inception. The Company concluded that these amounts do not require a constraint and are included in the transaction price at inception. The Company considers this estimate at each reporting date and updates the estimate based on information available. During the fourth quarter of 2021, the Company revised the estimate of the expected reimbursement to approximately $80.0 million based on expectations as a result from decisions made at the JSC meeting held in the fourth quarter of 2021, which resulted in significantly less research and development services to be provided by the Company under the 2019 Neurocrine Collaboration Agreement. During the fourth quarter of 2022, the Company further revised the estimate of the expected reimbursement to approximately $81.7 million, based on expectations resulting from decisions made at the JSC meeting held in the fourth quarter of 2022. Additional consideration to be paid to the Company upon reaching certain milestones are excluded from the transaction price at inception due to the uncertainty of achieving the development and regulatory milestones.

The Company allocated the fixed transaction price to the separate performance obligations based on the relative standalone selling price of each performance obligation or in the case of certain variable consideration to one or more performance obligations. The estimated standalone selling prices for performance obligations, which include a license and research services, were developed using the estimated selling price of the license, using comparable and market data, and an estimate of the overall effort to perform the research services along with a reasonable profit for research services.

The total variable consideration allocated to each program related to the expected cost reimbursement was as follows as of December 31, 2022:

Performance Obligation	Amount (in thousands)
Variable Consideration	
VY-AADC Program	$ 53,863
FA Program	18,868
2019 Discovery Program 1	5,336
2019 Discovery Program 2	3,605
Total	$ 81,671

Based on the relative standalone selling price allocation, the allocation of the transaction price, exclusive of the variable consideration allocated to the individual performance obligations, to the separate performance obligations was as follows:

Performance Obligation	Amount (in thousands)
Fixed Consideration	
VY-AADC Program	$ 49,045
FA Program	20,647
2019 Discovery Program 1	14,443
2019 Discovery Program 2	8,247
Total	$ 92,382

The Company recognizes the transaction price associated with each performance obligation on a proportional performance basis over the period of service using input-based measurements such as costs incurred to date, to estimate proportion performed, and remeasures its progress towards completion at the end of each reporting period.

The Company determined the partial termination of the 2019 Neurocrine Collaboration Agreement with respect to the VY-AADC Program represented a modification of the arrangement under ASC 606 and that the remaining fixed transaction price at the Neurocrine VY-AADC Program Termination Effective Date of $42.2 million should be re-allocated to the FA Program and 2019 Discovery Program 1 and 2 based on their standalone selling prices. Accordingly, the Company recorded a cumulative adjustment to revenue of approximately $0.9 million on the partially satisfied remaining performance obligations, as the remaining services to be performed under each of the performance obligations are not distinct from the services prior to the modification. The Company determined that reasonable changes to the Company's estimates of standalone selling prices for the FA Program, 2019 Discovery Program 1 and 2019 Discovery Program 2 performance obligations did not have a material impact on the re-allocation or the amount of revenue recorded pursuant to the cumulative catch-up adjustment.

During the years ended December 31, 2022 and 2021, the Company recognized $0.9 million and $37.4 million of revenue, respectively, associated with its collaboration with Neurocrine related to research and development services performed during the period and the corresponding cost reimbursement receivable. As of December 31, 2022, there was $11.8 million of deferred revenue related to the 2019 Neurocrine Collaboration Agreement, which is classified as either current or non-current in the accompanying consolidated balance sheet based on the period the services are expected to be delivered. Additionally, as of December 31, 2022, there was $0.3 million of collaboration receivables related to reimbursable costs expected to be received from Neurocrine for research and development services performed.

The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities during the year ended December 31, 2022:

	Balance at December 31, 2021	Additions	Deductions	Balance at December 31, 2022
		(*in thousands*)		
Related party collaboration receivable	$ 732	$ 907	$ (1,382)	$ 257
Contract liabilities:				
Deferred revenue	$ 12,096	$ —	$ (269)	$ 11,827

The change in the receivables balance for the year ended December 31, 2022 is primarily driven by amounts owed to the Company for research and development services provided, offset by amounts collected from Neurocrine during the period.

Costs incurred relating to the Company's collaboration programs under the 2019 Neurocrine Collaboration Agreement consist of internal and external research and development costs, which primarily include: salaries and benefits, laboratory supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's consolidated statements of operations.

The Company incurred approximately $0.8 million of costs to obtain the 2019 Neurocrine Collaboration Agreement which were payable only upon the close of the deal and therefore considered incremental costs of obtaining a contract with a customer and capitalized. The costs are recorded in prepaid expenses and other non-current assets and are being amortized over the period in which the research services will be provided.

Pfizer Option and License Agreement

Summary of Agreement

On October 1, 2021, the Company entered into an option and license agreement with Pfizer (the "Pfizer Agreement"), pursuant to which the Company granted Pfizer options to receive an exclusive license (the "Pfizer License Options") to certain TRACER capsids to develop and commercialize certain AAV gene therapy candidates comprised of a capsid and specified Pfizer transgenes (the "Pfizer Transgenes"). Under the terms of the Pfizer Agreement, during an initial research term that ended as of October 1, 2022 (the "Pfizer Research Term"), Pfizer had the right to evaluate the potential use of the capsids in combination with up to two Pfizer Transgenes to help treat respective central nervous system ("CNS") and cardiovascular diseases.

During the Pfizer Research Term, the Company agreed to provide Pfizer with certain quantities of materials encoding specified existing capsids for Pfizer's evaluation. Further, during the Pfizer Research Term, the Company agreed to disclose to Pfizer, on a rolling basis, the performance characteristics identified during the Pfizer Research Term for all such capsid candidates. Pfizer had the right, in its sole discretion, to select any capsid candidate for evaluation to determine its interest in exercising a Pfizer License Option with respect to such capsid candidate. Pfizer had the right to exercise up to two Pfizer License Options, provided that it could exercise only one Pfizer License Option for each Pfizer Transgene.

Effective as of September 30, 2022, Pfizer exercised its Pfizer License Option with respect to a capsid for the specified Pfizer Transgene for potential treatment of a rare neurological disease. Pfizer did not exercise its option to license a capsid for the potential treatment of a cardiovascular disease. As result, Pfizer's right to exercise a Pfizer License Option for a cardiovascular disease has terminated in accordance with the terms of the Pfizer Agreement and all rights to capsids for that cardiovascular disease have reverted to the Company. Pfizer's exercise of a Pfizer License Option extends the Pfizer Research Term to October 1, 2024, during which period the Company may, at its sole discretion and expense, conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of the rare neurological disease associated with the exercise of the applicable Pfizer License Option.

Pursuant to the exercise of the Pfizer License Option, the Company granted Pfizer an exclusive, worldwide license, with the right to sublicense, under certain of the Company's intellectual property, the rights to develop and commercialize rare neurological disease products utilizing the capsid candidate and incorporating the corresponding Pfizer Transgene (the "Pfizer Licensed CNS Products"). Until October 1, 2024, while the Company is not obligated to conduct additional research activities to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of rare neurological diseases, it has agreed to continue to disclose to Pfizer, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Pfizer may, during the Pfizer Research Term, conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid it previously elected to license when it exercised the Pfizer License Option.

Under the Pfizer Agreement, Pfizer is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Pfizer Licensed CNS Products. Pfizer is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Pfizer Licensed CNS Product for which Pfizer has exercised its Pfizer License Option in (a) the United States and (b) at least one of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan (each of which is referred to as a "Pfizer Major Market Country"), subject to certain limitations. Pfizer is also required to use commercially reasonable efforts to commercialize each Pfizer Licensed CNS Product in the United States and at least one Pfizer Major Market Country where Pfizer or its designated affiliates or sublicensees has received regulatory approval for such Pfizer Licensed CNS Product, subject to certain limitations.

Under the terms of the Pfizer Agreement, Pfizer paid the Company an upfront payment of $30.0 million in October 2021. Following the exercise of the Pfizer License Option, Pfizer paid the Company a fee of $10.0 million and the Company is also eligible to receive specified development, regulatory, and commercialization milestone payments of up to an aggregate of $115.0 million for the first corresponding Pfizer Licensed CNS Product to achieve the corresponding milestone. On a Pfizer Licensed CNS Product-by-Pfizer Licensed CNS Product basis, the Company is also eligible to receive (a) specified sales milestone payments of up to an aggregate of $175.0 million per Pfizer Licensed CNS Product and (b) tiered, escalating royalties in the mid- to high-single-digit percentages of annual net sales of each Pfizer Licensed CNS Product. The royalties are subject to potential reductions in customary circumstances including patent claim expiration, payments for certain third-party licenses, and biosimilar market penetration, subject to specified limits.

Under the terms of the Pfizer Agreement, each of the Company and Pfizer owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the effective date of the Pfizer Agreement, or invented, developed, created, generated or acquired solely by or on behalf of such party after such effective date.

Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Pfizer Agreement and in the course of the Company's and Pfizer's activities under the Pfizer Agreement will follow inventorship under U.S. patent law. Subject to certain limitations and exceptions, the Company agreed (a) during the Pfizer Research Term, not to conduct any internal program or program on behalf of a third party that is directed to development or commercialization of any capsid candidates, or grant any third party or affiliate any right or license under the Company's rights in such capsid candidates to exploit any therapeutic product, in combination with any Pfizer Transgene in any indication for therapeutic, diagnostic and prophylactic human and veterinary use; and (b) after Pfizer's exercise of a Pfizer License Option, not to grant any third party or affiliate any right or license under the Company's patents to exploit any licensed capsid in combination with any Pfizer Transgene.

Unless earlier terminated, the Pfizer Agreement expires on the expiration of the last-to-expire royalty term with respect to all Pfizer Licensed CNS Products in all countries. Subject to a cure period, either party may terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Pfizer may also terminate the Pfizer Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, the occurrence of a violation of global trade control laws, or for the Company's noncompliance

with certain anti-bribery or anti-corruption covenants. Pfizer may also terminate the Pfizer Agreement, in whole or in part, for any or no reason upon ninety days' written notice to us.

Upon certain terminations for cause by Pfizer, the license that the Company has granted to Pfizer under the Pfizer Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Pfizer under such license had the Pfizer Agreement remained in effect would be substantially reduced.

Accounting Analysis

At inception, the Company determined the Pfizer Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services under the Pfizer Agreement, in accordance with ASC 606, and determined that the Pfizer Agreement contains two performance obligations consisting of two material rights, one for each of the Pfizer License Options. The Company concluded that each Pfizer License Option provides a material right as consideration for each option is less than the amount that the Company would otherwise have expected to receive outside the context of the contract. The promises at inception do not include the underlying goods or services that would be delivered upon exercise of the option, but rather represent the value to the customer of having the right to exercise the Pfizer License Option at the specified exercise fee. Upon the exercise of a Pfizer License Option, until October 1, 2024, while the Company is not obligated to conduct additional research activities upon option exercise to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases, it has agreed to continue to disclose to Pfizer, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and when available. Pfizer may, conduct additional evaluations of such capsid candidates and has the right to substitute any other capsid candidate for the capsid it previously elected to license when it exercised the Pfizer License Option. The Company determined that this promise to provide Pfizer the ability to evaluate and potentially substitute other capsid candidates for the capsid it previously elected to license when it exercised the Pfizer License Option, if and when available, is an additional performance obligation in the arrangement ("the Pfizer Substitution Right Performance Obligation").

The Company received a nonrefundable, upfront payment of $30.0 million as consideration under the Pfizer Agreement, which represents the transaction price at inception. Additional consideration to be paid to the Company upon exercise of the Pfizer License Option or upon reaching certain milestones are excluded from the transaction price as they relate to option fees and milestones that could only be achieved subsequent to an option exercise.

The Company allocated the transaction price to the Pfizer License Options based on their relative standalone selling prices. The estimated standalone selling price for each material right was based on an adjusted market assessment approach. The Company concluded that the market would be willing to pay an equal amount for each Pfizer License Option on a standalone basis. The Company reached this conclusion after considering (a) the downstream economics including option fees, milestones and royalties related to each Pfizer License Option being identical and (b) comparable market data. The Company determined the standalone selling price for the Pfizer Substitution Right Performance Obligation was insignificant to the allocation of the transaction price using the relative standalone selling price model and, accordingly, did not allocate any transaction price to the Pfizer Substitution Right Performance Obligation. This determination was supported by qualitative and quantitative assessments of the standalone selling price that considered the cost of identifying other potential capsid candidates and the likelihood of license substitution. As such, based on the relative standalone selling price for each of the two material rights, the allocation of the transaction price to the separate performance obligations was $15.0 million for each material right. The amount allocated to each material right was initially recorded as deferred revenue.

During the year ended December 31, 2022, the Company recognized $40.0 million in collaboration revenue related to the Pfizer Agreement. Of this $40.0 million, $25.0 million is attributable to the exercise of the first material right for the Pfizer License Option for a rare neurological disease and includes the option exercise fee of $10.0 million. The remaining $15.0 million is attributable to the expiration of the second material right associated with the Pfizer License Option for a cardiovascular disease.

Novartis Option and License Agreement

Summary of Agreement

On March 4, 2022 (the "Novartis Effective Date"), the Company entered into an option and license agreement with Novartis (the "Novartis Agreement"). Pursuant to the Novartis Agreement, the Company has granted Novartis options (the "Novartis License Options") to license TRACER capsids ("Novartis Licensed Capsids") for exclusive use with certain targets to develop and commercialize adeno-associated virus gene therapy candidates comprised of Novartis Licensed Capsids and payloads directed to such targets (the "Novartis Payloads").

During the period commencing on the Novartis Effective Date and ending on the first anniversary thereof or, in the event Novartis exercises a Novartis License Option, the third anniversary thereof, on a target-by-target basis (the "Novartis Research Term"), the Company has granted Novartis a non-exclusive research license to evaluate the Company's TRACER capsids for potential use, in combination with Novartis Payloads, in programs targeting three specified genes (the "Initial Novartis Targets"). Upon the payment of additional fees, Novartis may also assess the Company's TRACER capsids for use with up to two other targets (the "Additional Novartis Targets"), subject to certain conditions including that such target is not part of, or reasonably competitive with, the Company's current development programs (the Initial Novartis Targets and the Additional Novartis Targets collectively, the "Novartis Targets"). During the Novartis Research Term, as applicable, the Company may, at its sole discretion and expense, conduct further research activities to identify additional TRACER capsids. If the Company elects to do so, the Company has agreed to disclose performance characteristics of such new TRACER capsids to Novartis on a rolling basis.

During the applicable Novartis Research Term, Novartis may exercise up to three Novartis License Options—or up to five Novartis License Options if Novartis is evaluating the Additional Novartis Targets—in the aggregate, provided that Novartis may only exercise one Novartis License Option for each Novartis Target. Upon the exercise of any Novartis License Option, the Company has agreed to grant Novartis a target-exclusive, worldwide license, with the right to sublicense, under certain of the Company's intellectual property, the rights to develop and commercialize the applicable Novartis Licensed Capsid as incorporated into products containing the corresponding Novartis Payload (the "Novartis Licensed Products"). Upon the exercise of a Novartis License Option, the Company has agreed to provide certain additional know-how to enable Novartis to exploit the Novartis Licensed Capsid and the corresponding Novartis Payload for use in a Novartis Licensed Product. Novartis may, during the applicable Novartis Research Term but following the exercise of a Novartis License Option, conduct additional evaluation of the Company's capsid candidates and has the right to substitute any other TRACER capsid for a Novartis Licensed Capsid.

Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from the Company's TRACER capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. For more information, refer to Note 15 to the Company's consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Subject to the Company's disclosure obligations described above, the Company and Novartis have agreed to conduct their respective research and evaluation activities independently, with communications being managed by two alliance managers comprised of a designee from each of the Company and Novartis.

Under the Novartis Agreement, Novartis is solely responsible for, and has sole decision-making authority with respect to, development and commercialization of the Novartis Licensed Products. In the event Novartis exercises a Novartis License Option, Novartis is required to use commercially reasonable efforts to develop and obtain regulatory approval for at least one Novartis Licensed Product for each Novartis Target for which it has exercised a Novartis License Option in (a) the United States and (b) at least three of the following countries: the United Kingdom, France, Germany, Italy, Spain and Japan (each of which, a "Novartis Major Market Country"), subject to certain limitations. Novartis is also required to use commercially reasonable efforts to commercialize each Novartis Licensed Product in the United States and at least three Novartis Major Market Countries where Novartis or its designated affiliates or sublicensees has received regulatory approval for such Novartis Licensed Product, subject to certain limitations.

During the Novartis Research Term, the Company has agreed to provide plasmids to Novartis for the production of TRACER capsids for evaluation upon request. The Company has also granted Novartis a non-exclusive license, effective upon an exercise of a Novartis License Option and in addition to its options for target-exclusive licenses under certain of the Company's intellectual property described above, on a Novartis Licensed Capsid-by-Novartis Licensed Capsid basis, under certain of the Company's know-how to exploit the applicable Novartis Licensed Capsid as incorporated into Novartis Licensed Products containing the corresponding Novartis Payload.

Under the terms of the Novartis Agreement, Novartis paid the Company an upfront payment of $54.0 million. Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from the Company's TRACER capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. For more information, refer to Note 15 to the Company's consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Under the terms of the Novartis Agreement, each party owns the entire right, title, and interest in and to all patents or know-how controlled by such party and existing as of or before the Novartis Effective Date, or invented, developed, created, generated or acquired solely by or on behalf of such party after the Novartis Effective Date. Subject to certain specified exceptions, any patents and know-how that are invented or otherwise developed jointly by or on behalf of the parties during the term of the Novartis Agreement and in the course of the parties' activities under the Novartis Agreement will follow inventorship under U.S. patent law.

Subject to certain limitations and exceptions, the Company has agreed (a) during the Novartis Research Term, not to conduct any internal program or program on behalf of a third party that is directed to the development or commercialization of any Company's capsids, or grant any third party or affiliate any right or license under the Company's rights in such capsids, to exploit any therapeutic product containing a capsid in combination with a payload designed to have therapeutic effect on any of the Novartis Targets; and (b) after Novartis's exercise of any Novartis License Option, not to grant any third party or affiliate any right or license under the Company's patents to exploit any Novartis Licensed Capsid for the applicable Novartis Target.

Unless earlier terminated, the Novartis Agreement expires on the expiration of the last-to-expire royalty term with respect to all Novartis Licensed Products in all countries. Subject to a cure period, either party may terminate the Novartis Agreement, in whole or in part, subject to specified conditions, in the event of the other party's uncured material breach. Novartis may also terminate the Novartis Agreement, in whole or in part, subject to specified conditions, for the Company's insolvency, the occurrence of a violation of global trade control laws, or for the Company's non-compliance with certain anti-bribery or anti-corruption covenants. Novartis may terminate the Novartis Agreement, in whole or in part, for any or no reason upon ninety days' written notice to the Company.

Upon certain terminations for cause by Novartis, the licenses granted by the Company to Novartis under the Novartis Agreement shall become irrevocable and perpetual, and all milestone payments and royalties that would have otherwise been payable by Novartis under such licenses had the Novartis Agreement remained in effect would be substantially reduced.

Accounting Analysis

At inception, the Company determined the Novartis Agreement was a contract with a customer under ASC 606. The Company assessed the promised goods and services and determined that the Novartis Agreement contains three performance obligations consisting of three material rights, one for each of the Novartis License Options. The Company concluded that each Novartis License Option provides a material right as consideration for each option is less than the amount that the Company would otherwise have expected to receive outside the context of the contract. The promises at inception do not include the underlying goods or services that would be delivered upon exercise of the option, but rather represent the value to the customer of having the right to exercise the Novartis License Option at the specified exercise fee. Upon the exercise of a Novartis License Option, until March 4, 2025, while the Company is not obligated to conduct additional research activities upon any option exercise to identify additional proprietary capsids that may be useful for AAV gene therapies for the treatment of central nervous system or cardiovascular diseases, it has agreed to continue to disclose to Novartis, on a rolling basis, the performance characteristics identified for all such capsid candidates, if and

when available. Novartis may conduct additional evaluation of such capsid candidates and has the right to substitute any other capsid candidate for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option. The Company determined that this promise to provide Novartis the ability to evaluate and potentially substitute other capsid candidates for the Novartis Licensed Capsid it previously elected to license when it exercised the Novartis License Option, if and when available, is an additional performance obligation in the arrangement (the "Novartis Substitution Right Performance Obligation"). The Company concluded the options for Additional Novartis Targets are not material rights as the price reflects the standalone selling price of the options. The Company will therefore account for the options for Additional Novartis Targets separately, if and when exercised.

The Company received a nonrefundable, upfront payment of $54.0 million as consideration under the Novartis Agreement, which represents the transaction price at inception. Additional consideration to be paid to the Company upon exercise of the Novartis License Options or upon reaching certain milestones are excluded from the transaction price as they relate to option fees and milestones that could only be achieved subsequent to an option exercise.

The Company allocated the transaction price to the three material rights based on their relative standalone selling prices. The estimated standalone selling price for each material right was based on an adjusted market assessment approach. The Company concluded that the market would be willing to pay an equal amount for each Novartis License Option on a standalone basis. The Company reached this conclusion after considering (i) the downstream economics including option fees, milestones and royalties related to each Novartis License Option being identical and (ii) comparable market data. The Company determined the standalone selling price for the Novartis Substitution Right Performance Obligation was insignificant to the allocation of the transaction price using the relative standalone selling price model and did not allocate any transaction price to the Novartis Substitution Right Performance Obligation, accordingly. This determination was supported by qualitative and quantitative assessments of the standalone selling price that considered the cost of identifying other potential capsid candidates and the likelihood of license substitution. As such, based on the relative standalone selling price for each of the three material rights, the allocation of the transaction price to the separate performance obligations is $18.0 million for each material right.

The amount allocated to each material right was recorded as deferred revenue and was recognized upon the exercise of two Novartis License Options in 2023 and upon the expiration of the remaining Novartis License Option in 2023.

During the year ended December 31, 2022, the Company did not recognize any revenue related to the Novartis Agreement. As of December 31, 2022, the entire transaction price of $54.0 million is recorded as deferred revenue, current in the accompanying consolidated balance sheet.

License Agreement with Touchlight IP Limited

On November 3, 2022, the Company and Touchlight IP Limited ("Touchlight") entered into a license agreement (the "Touchlight License Agreement") to authorize historical use by the Company of a certain DNA preparation process ("Subject DNA Preparation Process"), and to authorize the prospective exploitation of TRACER Capsids created with the use of the Subject DNA Preparation Process.

The terms of the Touchlight License Agreement include a one-time, non-refundable technology access fee of $5.0 million, which was paid during the fourth quarter of 2022. The Company recorded the $5.0 million to research and development expense in the year ended December 31, 2022, accordingly.

The terms of the Touchlight License Agreement also include future milestone payments and low single-digit royalties payable to Touchlight if the Company or its program collaborators or licensees choose to utilize in a therapeutic product TRACER Capsids that were created with the historical use of the Subject DNA Preparation Process. Additionally, the Company is obligated to pay low single-digit royalties to Touchlight on future payments the Company receives in connection with licensing of TRACER capsids that were created with the historical use of the Subject DNA Preparation Process, excluding the licensing of or collaboration on any Company therapeutic programs. No milestone or royalty payments were due and payable as of December 31, 2022.

Other Agreements

During the year ended December 31, 2016, the Company entered into a research and development funding arrangement with a non-profit organization that provides up to $4.0 million in funding to the Company upon the achievement of clinical and development milestones. The agreement provides that the Company repay amounts received under certain circumstances including termination of the agreement, and to pay an amount up to 2.6 times the funding received upon successful development and commercialization of any products developed. During the year ended December 31, 2017, the Company earned a milestone payment of $1.0 million. The Company evaluated the arrangement and concluded that it represents a research and development financing arrangement as it is probable that the Company will repay amounts received under the arrangement. As a result, the $1.0 million for the year ended December 31, 2017 is recorded as a non-current liability in the consolidated balance sheet.

Litigation

The Company was not a party to any material legal matters or claims and did not have contingency reserves established for any litigation liabilities as of December 31, 2022 or 2021.

10. Common stock

As of December 31, 2022 and 2021, the Company had authorized 120,000,000 shares of common stock, at $0.001 par value per share.

General

The voting, dividend and liquidation rights of the holders of the common stock are subject to and qualified by the rights, powers and preferences of the holders of preferred stock. The common stock has the following characteristics:

Liquidation

The holders of shares of common stock are entitled to share ratably in the Company's remaining assets available for distribution to its stockholders in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon occurrence of a deemed liquidation event.

Shares Reserved For Future Issuance

	As of December 31,	
	2022	2021
Shares reserved for vesting of restricted stock awards under the Founder Agreements	45,000	137,255
Shares reserved for exercise of outstanding stock options	6,199,571	5,013,193
Shares reserved for vesting of outstanding restricted stock units	1,112,563	806,379
Shares reserved for issuances under the 2015 Stock Option Plan	3,536,932	4,374,539
Shares reserved for issuances under the 2015 Employee Stock Purchase Plan	1,884,309	1,659,574
	12,778,375	11,990,940

11. Stock-based compensation

2014 Stock Option and Grant Plan

In January 2014, the Company adopted the 2014 Stock Option and Grant Plan (the "2014 Plan"), under which it could grant incentive stock options, non-qualified stock options, restricted stock awards, unrestricted stock awards, or restricted stock units to purchase up to 823,529 shares of common stock to employees, officers, directors and consultants of the Company.

The terms of stock option agreements, including vesting requirements, were determined by the Board of Directors and were subject to the provisions of the 2014 Plan. Restricted stock awards granted by the Company generally vest based on each grantee's continued service with the Company during a specified period following grant. Stock options granted to employees generally vest over four years, with 25% vesting on the one year anniversary and 75% vesting ratably, on a monthly basis, over the remaining three years. Stock options granted to non-employee consultants generally vest monthly over a period of one to four years.

Founder Awards

In January 2014, the Company issued 1,188,233 shares of restricted stock to its founders (each, a "Founder") at an original issuance price of $0.0425 per share. Of the total restricted shares awarded to the Founders, 835,292 shares were slated to vest over one to four years, based on each Founder's continued service to the Company in varying capacity as a Scientific Advisory Board member, consultant, director, officer or employee, as set forth in each grantee's individual restricted stock purchase agreement.

The remainder of the restricted stock awards were slated to vest upon the achievement of certain performance objectives as well as continued service to the Company, as set forth in the agreements. Stock-based compensation expense associated with these performance-based awards is recognized when the achievement of the performance condition is considered probable, using management's best estimates. The Company has modified certain of the awards, including repurchasing a total of 131,470 shares underlying the awards through December 31, 2022, and modifying the vesting provisions such that the modified awards vest over time rather than based on performance. The stock-based compensation expense recorded related to these awards during the years ended December 31, 2022, 2021, and 2020 were immaterial to the Company's consolidated financial statements.

2015 Stock Option Plan

In October 2015, the Company's board of directors and stockholders approved the 2015 Stock Option and Incentive Plan ("2015 Stock Option Plan"), which became effective upon the completion of the IPO. The 2015 Stock Option Plan provides the Company with the flexibility to use various equity-based incentive and other awards as compensation tools to motivate its workforce. These tools include stock options, stock appreciation rights, restricted stock, restricted stock units, unrestricted stock, performance share awards and cash-based awards. The 2015 Stock Option Plan replaced the 2014 Plan. Any options or awards outstanding under the 2014 Plan remained outstanding and effective. The number of shares initially reserved for issuance under the 2015 Stock Option Plan is the sum of (a) 1,311,812 shares of common stock and (b) the number of shares under the 2014 Plan that are not needed to fulfill the Company's obligations for awards issued under the 2014 Plan as a result of forfeiture, expiration, cancellation, termination or net issuances of awards thereunder. The number of shares of common stock that may be issued under the 2015 Stock Option Plan is also subject to increase on the first day of each fiscal year by up to 4% of the Company's issued and outstanding shares of common stock on the immediately preceding December 31.

Effective January 1, 2016 and every anniversary thereafter an additional 4% of outstanding common stock was added to the Company's 2015 Stock Option Plan pursuant to its "evergreen" provision, for future issuance. This has accumulated to a total of 10,771,368 shares through January 1, 2023. During the year ended December 31, 2022, the Company granted options to purchase 3,291,075 shares of common stock to employees and directors under the 2015 Stock Option Plan. As of December 31, 2022, there were 3,536,932 shares available for future issuance under the 2015 Stock Option Plan.

2015 Employee Stock Purchase Plan

In October 2015, the Company's board of directors and stockholders approved the 2015 Employee Stock Purchase Plan (the "2015 ESPP"). Under the 2015 ESPP, all full-time employees of the Company are eligible to purchase common stock of the Company twice per year, at the end of each six-month payment period. During each payment period, eligible employees who so elect, may authorize payroll deductions in an amount of 1% to 10% (whole percentages only) of the employee's base pay for each payroll period. At the end of each payment period, the

accumulated deductions are used to purchase shares of common stock from the Company at a discount. A total of 262,362 shares of common stock were initially authorized for issuance under this plan.

The 2015 ESPP became effective upon the completion of the IPO. Effective January 1, 2016 and every anniversary thereafter an additional 1% of outstanding common stock was added to the 2015 ESPP, pursuant to its evergreen provision, for future issuance. This has accumulated to a total of 2,692,838 shares through January 1, 2023. The Company issued 150,265 and 200,006 shares of common stock under the 2015 ESPP in the years ended December 31, 2022 and 2021. As of December 31, 2022, there were 1,884,309 shares available for future purchase under the 2015 ESPP.

Inducement Awards

In the years ended December 31, 2022, 2021, and 2020, the Company issued non-statutory stock options to purchase an aggregate of 390,000, 76,500 and 172,500 shares of the Company's common stock and restricted stock unit awards for an aggregate of 163,000, 13,000 and 29,000 shares of the Company's common stock, respectively, in each case outside of the Company's 2015 Stock Option Plan as an inducement material to certain individuals' acceptance of an offer of employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4).

The stock options will vest over a four-year period, with 25% of the shares underlying the option award vesting on the first anniversary of the award and the remaining 75% of the shares underlying the award vesting monthly thereafter over the subsequent 36-month period. The restricted stock units vest over a three-year period, with 33% of the restricted stock units vesting on the first anniversary of the award, 33% of the restricted stock units vesting on the second anniversary, and the remaining restricted stock units vesting on the third anniversary.

Stock-based Compensation Expense

Total compensation cost recognized for all stock-based compensation awards in the statements of operations and comprehensive (loss) income is as follows:

	Year ended December 31,		
	2022	2021	2020
	(in thousands)		
General and administrative	$ 6,398	$ 7,191	$ 8,577
Research and development	2,946	4,133	6,357
Total stock-based compensation expense	$ 9,344	$ 11,324	$ 14,934

Stock-based compensation expense by type of award included within the consolidated statements of operations and comprehensive (loss) income was as follows:

	Year ended December 31,		
	2022	2021	2020
	(in thousands)		
Stock options	$ 5,938	$ 7,438	$ 11,387
Restricted stock awards and units	3,215	3,551	3,110
Employee stock purchase plan awards	191	335	437
Total stock-based compensation expense	$ 9,344	$ 11,324	$ 14,934

Restricted Stock Units

A summary of the status of and changes in unvested restricted stock unit activity under the Company's equity award plans for the year ended December 31, 2022 was as follows:

	Units	Weighted Average Grant Date Fair Value Per Unit
Unvested restricted stock units as of December 31, 2021	806,379	$ 7.26
Awarded	964,480	$ 4.29
Vested	(436,611)	$ 6.92
Forfeited	(221,685)	$ 5.05
Unvested restricted stock units as of December 31, 2022	1,112,563	$ 5.27

Stock-based compensation of restricted stock units is based on the fair value of the Company's common stock on the date of grant and recognized over the vesting period. In the year ended December 31, 2022, the Company granted 864,480 restricted stock units vesting in equal amounts, annually over three years, and 100,000 restricted stock units vesting in equal amounts, annually over four years. The stock-based compensation expense was $2.9 million, $3.3 million, and $2.8 million for the years ended December 31, 2022, 2021, and 2020, respectively.

As of December 31, 2022, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock units of $3.7 million which is expected to be recognized over the remaining weighted average vesting period of 1.9 years.

Stock Options

A summary of the status of, and changes in, stock options was as follows:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2021	5,013,193	$ 12.69		
Granted	3,681,075	$ 5.17		
Exercised	(89,012)	$ 7.07		
Cancelled or forfeited	(2,405,685)	$ 13.15		
Outstanding at December 31, 2022	6,199,571	$ 8.12	7.9	$ 6,095
Exercisable at December 31, 2022	2,744,489	$ 10.56	6.5	$ 2,287

Using the Black-Scholes option pricing model, the weighted average fair value of options granted during the year ended December 31, 2022 was $3.60. The stock-based compensation expense related to stock option awards granted was $5.8 million, $7.3 million, and $11.2 million for the years ended December 31, 2022, 2021, and 2020, respectively.

The fair value of each option was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,		
	2022	2021	2020
Risk-free interest rate	2.2 %	0.9 %	1.0 %
Expected dividend yield	— %	— %	— %
Expected term (in years)	6.0	6.0	6.0
Expected volatility	79.4 %	75.0 %	73.7 %

As of December 31, 2022, the Company had unrecognized stock-based compensation expense related to its unvested stock options of $12.8 million which is expected to be recognized over the remaining weighted average vesting period of 2.9 years.

12. 401(k) Savings plan

The Company has a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pretax basis. The Company expensed approximately $0.9 million, $1.1 million, and $1.1 million related to employer contributions made during the years ended December 31, 2022, 2021, and 2020, respectively.

13. Income taxes

The Company recognized deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the Company's financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on differences between the financial statement carrying amounts and the tax bases of the assets and liabilities using the enacted tax rates in effect in the years in which the differences are expected to reverse. A valuation allowance against deferred tax assets is recorded if, based on the weight of the available evidence, it is more likely than not that some or all the deferred tax assets will not be realized. The Company accounts for uncertain tax positions using a more-likely-than-not threshold for recognizing and resolving uncertain tax positions. The evaluation of uncertain tax positions is based on factors including, but not limited to, changes in the law, the measurement of tax positions taken or expected to be taken in tax returns, the effective settlement of matters subject to audit, new audit activity, and changes in facts or circumstances related to a tax position. The Company evaluates its tax positions on an annual basis. The provision for incomes taxes is as follows:

	Year ended December 31,		
	2022	2021	2020
	(in thousands)		
Current			
Federal	$ —	$ —	$ —
State	16	—	—
Total current	16	—	—
Deferred			
Federal	—	—	—
State	—	—	—
Total deferred	—	—	—
Total tax provision	$ 16	$ —	$ —

A reconciliation of the expected income tax provision computed using the federal statutory income tax rate at the Company's effective tax rate for the years ended December 31, 2022, 2021, and 2020 is as follows:

	Year ended December 31,		
	2022	2021	2020
Income tax computed at federal statutory tax rate	21.0 %	21.0 %	21.0 %
State taxes, net of federal benefit	5.2 %	6.6 %	(2.3)%
Provision to return	3.2 %	4.9 %	— %
General business credit carryovers	(3.5)%	3.2 %	(20.6)%
Non-deductible expenses	(4.6)%	(3.8)%	5.0 %
Other	— %	— %	1.9 %
Change in valuation allowance	(21.3)%	(31.9)%	(5.0)%
Total	— %	— %	— %

The Company has historically incurred net operating losses ("NOLs"). As of December 31, 2022, the Company had federal and state net operating loss carryforwards of $175.1 million and $166.5 million, respectively. As of December 31, 2022, the Company had federal and state research and development tax credit carryforwards of $24.0 million and $9.6 million, respectively, which expire beginning in 2033. As of December 31, 2021, the Company had state investment credits of $0.5 million, which expire beginning in 2023.

The significant components of the Company's deferred tax assets and (liabilities) as of December 31, 2022 and 2021 are as follows:

	As of December 31,	
	2022	2021
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforward	$ 47,282	$ 56,756
Tax credit carryforward	32,060	30,122
Lease liability	6,318	12,012
Deferred revenue	17,984	11,485
Stock compensation	4,630	7,784
Non-deductible accruals and reserves	1,603	1,507
Capitalized research expenses	14,351	—
Intangibles	610	664
Other temporary differences	(1)	—
Total deferred tax assets	124,837	120,330
Less valuation allowance	(117,416)	(107,563)
Net deferred tax assets	7,421	12,767
Deferred tax liabilities		
Right of use assets	(4,231)	(9,128)
Depreciation and amortization	(3,190)	(3,632)
Other temporary differences	—	(7)
Net deferred taxes	$ —	$ —

As required by ASC 740, management has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which principally comprise NOL carryforwards, research and development credit carryforwards, and lease liability. Management has determined that it is more likely than not that the Company will not recognize the benefits of its federal and state deferred tax assets, and as a result, a valuation allowance of $117.4 million and $107.6 million has been established at December 31, 2022 and 2021, respectively. The change in valuation allowance was $9.8 million for the year ended December 31, 2022. The primary reason for the difference between the income tax provision recorded by the Company and the amount of income tax provision at statutory income tax rates was the change in the valuation allowance.

At December 31, 2022 and 2021, the Company had no unrecognized tax benefits. The Company has not as yet conducted a study of its research and development credit carryforwards. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed, and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits, and if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the balance sheets or statements of operations if an adjustment were required.

Interest and penalty charges, if any, related to unrecognized tax benefits would be classified as income tax expense in the accompanying statements of operations. As of December 31, 2022 and 2021, the Company has no accrued interest related to uncertain tax positions. Since the Company is in a loss carryforward position, it is generally subject to examination by the U.S. federal, state, and local income tax authorities for all tax years in which a loss carryforward is available.

14. Related-party transactions

During the years ended December 31, 2022, 2021, and 2020, the Company received board and scientific advisory services from two of its prior executives, Steven M. Paul, M.D., the Company's former President and Chief Executive Officer, and Dinah Sah, Ph.D., the Company's former Chief Scientific Officer. As of December 31, 2020, Dr. Paul's consulting agreement was complete. The total amount of fees paid to Dr. Paul for services provided during the year ended December 31, 2020 was $0.2 million. The total amount of fees paid to Dr. Sah for services provided during the years ended December 31, 2022, 2021 and 2020 was $0.5 million, $0.2 million, and $0.4 million, respectively.

During the year ended December 31, 2022, the Company received advisory services related to strategic planning, operations, and management from Alfred Sandrock, M.D., Ph.D., the Company's current President and Chief Executive Officer and a member of the Company's Board of Directors, before he commenced service in the capacity of President and Chief Executive Officer in March 2022. The total amount of fees paid to Dr. Sandrock for services provided was $60,000 for the year ended December 31, 2022.

Under the 2019 Neurocrine Collaboration Agreement, the Company and Neurocrine have agreed to conduct research, development and commercialization activities for certain of the Company's AAV gene therapy products (Note 9). Amounts due from Neurocrine are reflected as related party collaboration receivables. As of December 31, 2022, the Company recorded approximately $0.3 million in related party collaboration receivables.

15. Subsequent events

2023 Neurocrine Collaboration

On January 8, 2023, the Company entered into the 2023 Neurocrine Collaboration Agreement, with Neurocrine for the research, development, manufacture and commercialization of gene therapy products directed to the gene that encodes glucosylceramidase beta 1 ("GBA1") for the treatment of Parkinson's disease and other diseases associated with GBA1 (the "GBA1 Program") and three new programs focused on the research, development, manufacture and commercialization of gene therapies designed to address central nervous system diseases or conditions associated with rare genetic targets (the "2023 Discovery Programs" and, collectively with the GBA1 Program, the "2023 Neurocrine Programs").

Under the terms of the 2023 Neurocrine Collaboration Agreement, Neurocrine paid to the Company an upfront payment of approximately $136.0 million (the "Upfront Collaboration Payment") and approximately $39.0 million as consideration (the "Share Consideration") for an equity purchase of 4,395,588 shares of the Company's common stock (the "Shares") in February 2023. The 2023 Collaboration Agreement also provides for aggregate development milestone payments from Neurocrine for gene therapy products arising under the 2023 Neurocrine Programs (the "2023 Collaboration Products") under (a) the GBA1 Program of up to $985.0 million; and (b) each of the three 2023 Discovery Programs of up to $175.0 million for each 2023 Discovery Program. The Company may be entitled to receive aggregate commercial milestone payments for up to two 2023 Collaboration Products under the GBA1 Program of up to $950.0 million per 2023 Collaboration Product and for one 2023 Collaboration Product under each 2023 Discovery Program of up to $275.0 million per 2023 Discovery Program.

The 2023 Neurocrine Collaboration Agreement became effective on February 21, 2023, upon expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended. On February 23, 2023, the Company received the Upfront Collaboration Agreement and the Share Consideration and issued and sold to Neurocrine the Shares pursuant to the applicable stock purchase agreement.

In connection with the execution of the 2023 Neurocrine Collaboration Agreement, the Company amended and restated their existing investor agreement on January 8, 2023 (the "2023 Neurocrine Amended and Restated Investor Agreement"), providing for standstill and lock-up restrictions and a voting agreement with respect to shares of the Company owned by Neurocrine.

Novartis Option Exercises

Effective as of March 1, 2023, Novartis exercised its Novartis License Options to license novel capsids generated from the Company's TRACER capsid discovery platform for use in gene therapy programs against two undisclosed Initial Novartis Targets. With Novartis' option exercise on two Initial Novartis Targets, the Company will receive a $25.0 million option exercise payment during the first half of 2023, and is eligible to receive associated potential development, regulatory, and commercial milestone payments, as well as mid- to high-single-digit tiered royalties based on net sales of the Novartis Licensed Products incorporating the Novartis Licensed Capsids. The two Initial Novartis Targets licensed are distinct from targets in the Company's internal and partnered pipeline. In addition, over the next 18 months, Novartis retains the right to expand the agreement to include options to license capsids for up to two Additional Novartis Targets, subject to their availability, for a fee of $18.0 million per Additional Novartis Target. Under such an expansion, the Company would be eligible to receive a $12.5 million license option exercise fee for each Additional Novartis Target exercised, as well as future potential milestone payments per Additional Novartis Target and tiered mid- to high-single digit royalties on the Novartis Licensed Products incorporating the Novartis Licensed Capsids.

Novartis elected not to license a capsid for one Initial Novartis Target under the Novartis Agreement prior to the expiration of the applicable Novartis License Option. As a result, the non-exclusive research license that we granted to Novartis in connection with this Initial Novartis Target has terminated, the Novartis Research Term for this Initial Novartis Target has expired, and we are no longer eligible to receive development, regulatory, and commercial milestone payments or royalties in connection with this Initial Novartis Target. All capsid rights with respect to that Initial Novartis Target have returned to the Company.

EXHIBIT INDEX

Exhibit No.	Description	Incorporated by Reference to:				Filed Herewith
		Form or Schedule	Exhibit No.	Filing Date with SEC	SEC File Number	
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	3.1	11/16/2015	001-37625	
3.2	Amended and Restated By-Laws of the Registrant	8-K	3.2	11/16/2015	001-37625	
4.1	Specimen Common Stock Certificate of the Registrant	10-K	4.1	03/14/2018	001-37625	
4.4	Description of Registrant's Securities	10-K	4.4	03/03/2020	001-37625	
10.1#	2014 Stock Option and Grant Plan and forms of award agreements thereunder	S-1/A	10.1	10/28/2015	333-207367	
10.2#	2015 Stock Option and Incentive Plan and forms of award agreements thereunder	S-1/A	10.2	10/28/2015	333-207367	
10.3†	Collaboration Agreement, by and between the Registrant and Sanofi Genzyme Corporation, dated February 11, 2015	S-1/A	10.3	11/06/2015	333-207367	
10.4*	Termination Agreement, by and between the Registrant and Genzyme Corporation, dated June 14, 2019	10-Q	10.3	08/09/2019	001-37625	
10.5*	Amended and Restated Option and License Agreement, by and between the Registrant and Genzyme Corporation, dated June 14, 2019	10-Q	10.4	08/09/2019	001-37625	
10.6*	First Amendment to Amended and Restated Option and License Agreement with Genzyme Corporation, dated September 20, 2020	10-Q	10.1	11/09/2020	001-37625	
10.7†	Collaboration and License Agreement, by and between the Registrant and Neurocrine Biosciences, Inc., dated January 28, 2019	10-K	10.28	02/26/2019	001-37625	
10.8	Amendment No. 1 to the Collaboration and License Agreement, by and between the Registrant and Neurocrine Biosciences, Inc., dated June 14, 2019	10-Q	10.5	08/09/2019	001-37625	
10.09*	Option and License Agreement, by and between the Registrant and Pfizer Inc., dated October 1, 2021	10-Q	10.2	11/02/21	001-37625	

10.10	Lease Agreement, by and between the Registrant and UP 45/75 Sidney Street, LLC, dated April 1, 2014	S-1/A	10.5	10/28/2015	333-207367
10.11	First Amendment to the Lease Agreement, by and between the Registrant and UP 45/75 Sidney Street, LLC, dated December 23, 2015	10-Q	10.5	05/12/2016	001-37625
10.12	Second Amendment to the Lease Agreement, by and between the Registrant and UP 45/75 Sidney Street, LLC, dated February 5, 2018	8-K	10.1	02/07/2018	001-37625
10.13	Third Amendment to the Lease Agreement, by and between the Registrant and UP 45/75 Sidney Street, LLC, dated June 1, 2018	8-K	10.1	06/05/2018	001-37625
10.14	Sublease Agreement, by and between Registrant and BioNTech US Inc., dated September 3, 2021	10-Q	10.1	11/02/2021	001-37625
10.15	Lease Agreement, by and between the Registrant and UP 64 Sidney Street, LLC, dated December 23, 2015	10-Q	10.6	05/12/2016	001-37625
10.16	First Amendment to the Lease Agreement, by and between the Registrant and UP 64 Sidney Street, LLC, dated June 1, 2018	8-K	10.2	06/05/2018	001-37625
10.17	Lease Agreement, by and between the Registrant and HCP/King 75 Hayden LLC, dated March 16, 2020	8-K	10.1	03/19/2020	001-37625
10.18	Form of Indemnification Agreement to be entered into between the Registrant and its directors	S-1/A	10.9	10/28/2015	333-207367
10.19	Form of Indemnification Agreement to be entered into between the Registrant and its executive officers	S-1/A	10.10	10/28/2015	333-207367
10.20#	2015 Employee Stock Purchase Plan	S-1/A	10.12	10/28/2015	333-207367
10.21#	Amendment No. 1 to the 2015 Employee Stock Purchase Plan	10-K	10.21	03/14/2018	001-37625
10.22#	Retirement Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated May 20, 2019	8-K	10.1	05/21/2019	001-37625

10.23#	Employment Agreement, by and between the Registrant and Michael Higgins, dated May 19, 2021	8-K	10.2	05/19/2021	001-37625
10.24#	Employment Agreement, by and between the Registrant and Glenn Pierce, M.D., Ph.D., dated May 19, 2021	8-K	10.3	05/19/2021	001-37625
10.25#	Amendment No. 1 to Employment Agreement, by and between the Registrant and Glenn Pierce, dated June 7, 2021	8-K	10.1	06/08/2021	001-37625
10.26#	Employment Agreement, by and between the Registrant and Robert W. Hesslein, dated January 15, 2019	10-Q	10.5	05/07/2019	001-37625
10.27#	Amended and Restated Employment Agreement, by and between the Registrant and Robin Swartz, effective as of February 7, 2022	8-K	10.2	02/03/2022	001-37625
10.28#	Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated June 28, 2019	10-Q	10.6	08/09/2019	001-37625
10.29#	Amendment No. 1 to the Consulting Agreement, by and between the Registrant and Dinah Sah, Ph.D., dated September 16, 2019	10-Q	10.2	11/06/2019	001-37625
10.30#	Consulting Agreement by and between the Registrant and Alfred Sandrock, effective as of February 7, 2022	8-K	10.1	02/03/2022	001-37625
10.31#	Form of Non-Qualified Stock Option Agreement for Inducement	10-K	10.27	02/26/2019	001-37625
10.32#	Form of Restricted Stock Unit Agreement for Inducement	10-K	10.33	02/26/2019	001-37625
10.33	Sales Agreement, by and between the Registrant and Cowen and Company, LLC, dated November 8, 2022	S-3	1.2	11/08/2022	333-268240
10.34#	Consulting Agreement by and between the Registrant and Allison Dorval, dated as of November 26, 2021	10-K	10.38	03/08/2022	001-37625
10.35*	Consulting Agreement by and between the Registrant and Alfred Sandrock, effective as of February 7, 2022	8-K	10.1	02/03/2022	001-37625

10.36	Option and License Agreement by and between the Registrant and Novartis Pharma AG, dated March 4, 2022					X
10.37#	Employment Agreement, by and between the Registrant and Alfred Sandrock, M.D., Ph.D., effective as of March 22, 2022.	8-K	10.1	03/22/2022	001-37625	
10.38#	Consulting Agreement by and between the Registrant and Glenn Pierce, M.D., Ph.D., effective as of June 6, 2022	8-K	10.1	06/07/2022	001-37625	
10.39	Lease Termination Agreement by and between the Registrant and BRE-BMR Pilgrim & Sidney LLC, dated as of June 22, 2022	8-K	10.1	06/23/2022	001-37625	
10.40	Sublease Termination Agreement by and between the Registrant and BioNTech US, Inc., dated as of June 22, 2022	8-K	10.2	06/23/2022	001-37625	
10.41	Employment Agreement by and between the Registrant and Peter Pfreundschuh, effective as of September 7, 2022	8-K	10.1	09/07/2022	001-37625	
10.42	Second Amended and Restated Employment Agreement by and between the Registrant and Todd Carter, Ph.D., effective as of September 7, 2022	8-K	10.2	09/07/2022	001-37625	
10.43	Patent and Know-How Licence between the Registrant and Touchlight IP Limited, dated as of November 3, 2022					X
10.44	Stock Purchase Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023					X
10.45	Collaboration and License Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023					X
10.46	Amended and Restated Investor Agreement by and between the Registrant and Neurocrine Biosciences, Inc., dated as of January 8, 2023					X
10.47#	Transition, Separation and Release of Claims Agreement, by and between the Company and Robert W. Hesslein, dated February 22, 2023.	8-K	10.1	02/23/2023	001-37625	
21.1	Subsidiaries of the Registrant.					X

23.1	Consent of Ernst & Young, Independent Registered Public Accounting Firm.	X
24.1	Power of Attorney (see signature page of this Annual Report on Form 10-K).	X
31.1	Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.	X
31.2	Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.	X
32.1+	Certifications of Principal Executive Officer and Principal Financial Officer pursuant to Exchange Act Rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.	X
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Document.	X
101.LAB	Inline XBRL Taxonomy Extension Definition Linkbase Document.	X
101.PRE	Inline XBRL Taxonomy Extension Labels Linkbase Document.	X
101.DEF	Inline XBRL Taxonomy Extension Presentation Link Document.	X
104	Cover Page Interactive Data File – The cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document	

Management contract or compensatory plan or arrangement filed in response to Item 15(a)(3) of the Instructions to the Annual Report on Form 10-K.

† Confidential treatment has been granted as to certain portions, which portions have been omitted and separately filed with the Securities and Exchange Commission.

* Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K

+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Annual Report on Form 10-K and will not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

March 7, 2023

VOYAGER THERAPEUTICS, INC.
By: */s/ Alfred Sandrock, M.D., Ph.D.*
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director

SIGNATURES AND POWER OF ATTORNEY

We, the undersigned directors and officers of Voyager Therapeutics, Inc. (the "Company"), hereby severally constitute and appoint Alfred Sandrock and Peter Pfreundschuh, and each of them singly, our true and lawful attorneys, with full power to them, and to each of them singly, to sign for us and in our names in the capacities indicated below, any and all amendments to this Annual Report on Form 10-K, and to file or cause to be filed the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as each of us might or could do in person, and hereby ratifying and confirming all that said attorneys, and each of them, or their substitute or substitutes, shall do or cause to be done by virtue of this Power of Attorney.

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K has been signed by the following persons in the capacities and on the dates indicated.

Name	Title	Date
/s/Alfred Sandrock, M.D., Ph.D. Alfred Sandrock, M.D., Ph.D.	Chief Executive Officer, President, and Director *(Principal Executive Officer)*	March 7, 2023
/s/Peter P. Pfreundschuh Peter P. Pfreundschuh	Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 7, 2023
/s/Michael Higgins Michael Higgins	Director (Chairman of the Board)	March 7, 2023
/s/Grace E. Colón, Ph.D. Grace E. Colón, Ph.D.	Director	March 7, 2023
/s/Jim Geraghty Jim Geraghty	Director	March 7, 2023
/s/Steven Hyman, M.D. Steven Hyman, M.D.	Director	March 7, 2023
/s/Catherine J. Mackey, Ph.D. Catherine J. Mackey, Ph.D.	Director	March 7, 2023
/s/Jude Onyia, Ph.D. Jude Onyia, Ph.D.	*Director*	March 7, 2023
/s/Glenn Pierce, M.D., Ph.D. Glenn Pierce, M.D., Ph.D.	Director	March 7, 2023
/s/Nancy Vitale Nancy Vitale	Director	March 7, 2023

Exhibit 10.36

Execution Version
Confidential

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

OPTION AND LICENSE AGREEMENT

By and between

VOYAGER THERAPEUTICS, INC.

AND

NOVARTIS PHARMA, A.G.

March 4, 2022

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Schedule 1.23: Capsid Patents Covering Capsid Candidates as of the Effective Date

OPTION AND LICENSE AGREEMENT

This OPTION AND LICENSE AGREEMENT (the "Agreement") is entered into and made effective as of March 4, 2022(the "Effective Date"), by and between Voyager Therapeutics, Inc., a Delaware corporation, having its principal place of business at 75 Sidney Street, Cambridge, MA 02139 ("Voyager"), and Novartis Pharma AG, a corporation, having its principal place of business at Lichtstrasse 35, CH-4056 Basel, Switzerland ("Novartis"). Voyager and Novartis are referred to herein individually as a "Party" and collectively as the "Parties".

RECITALS

WHEREAS, Voyager Controls certain Patents, Know-How, scientific and technical information, and other proprietary rights and information relating to the generation and selection of Capsids (as defined below) for use in AAV Gene Therapy;

WHEREAS, Novartis is engaged in the research, development and commercialization of certain AAV Gene Therapies (as defined below), and desires to access certain Capsids developed by Voyager; and

WHEREAS, in furtherance of the foregoing, Voyager and Novartis are entering into this Agreement for Voyager to provide Novartis with access to Capsids discovered by Voyager prior to the Effective Date or discovered by Voyager after the Effective Date in its ongoing screening campaigns, and to provide Novartis with an option and license under Voyager's intellectual property rights to develop and commercialize Licensed Products in the Territory.

NOW, THEREFORE, in consideration of the premises and mutual covenants set forth in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

As used in this Agreement, the following terms will have the meanings set forth in this Article 1 or otherwise ascribed to them elsewhere in this Agreement:

1.1 "AAV" means an adeno-associated virus, including its recombinant forms.

1.2 "AAV Gene Therapy" means therapies and products that use a viral vector, including an AAV vector, to deliver nucleic acid(s) into a patient's cells to treat a human disease, syndrome, disorder, illness or condition.

1.3 "Accounting Standards" means in the case of Voyager, United States Generally Accepted Accounting Principles, and in the case of Novartis IFRS, (International Financial Reporting Standards), in each case as generally and consistently applied throughout the applicable Party's organization. Each Party shall promptly notify the other in the event that it changes the Accounting Standards pursuant to which its records are maintained, it being understood that each Party may only use internationally recognized accounting principles (e.g., IFRS, US GAAP, etc.).

1.4 "Acquiring Entity" has the meaning set forth in Section 1.30.

1.5 "Additional Target" means a Rare Disease Target, other than an Initial Target, that is: (a) Available at the time of nomination by Novartis pursuant to Section 2.2.3(a); and (b) for which Novartis has exercised an Additional Target Option under Section 2.2.3(b).

1.6 "Additional Target Option" has the meaning set forth in Section 2.2.3(a).

1.7 "Additional Target Option Period" means: (a) twelve (12) months after the Effective Date; or (b) if any Option is exercised for any Initial Target during the time period set forth in subsection (a), thirty (30) months after the Effective Date.

1.8 "Affiliate" means with respect to a Person, any other Person that (directly or indirectly) is controlled by, controls or is under common control with such Person as of any point in time and continuing for as long as such relationship continues to exist with respect to such other Person. For the purposes of this definition, the term "control" (including, with correlative meanings, the terms "controlled by" and "under common control with") as used with respect to a Person, will mean the possession, directly or indirectly, of the power to direct, or cause the direction of, the management or policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and "control" will be presumed to exist if either of the following conditions is met: (a) in the case of a corporate entity, direct or indirect ownership of voting securities entitled to cast at least 50% (or in the case of entities organized under the laws of certain countries where the maximum percentage ownership permitted by law for a foreign investor is less than fifty percent (50%), the maximum ownership interest permitted by applicable Law) of the voting securities or other ownership or general partnership interest (whether directly or pursuant to any option, warrant or other similar arrangement) or other comparable equity interests of an entity; *provided*, *however*, that where an entity owns a majority of the voting power necessary to elect a majority of the board of directors or other governing board of another entity, but is restricted from electing such majority by contract or otherwise, such entity will not be considered to be in control of such other entity until such time as such restrictions are no longer in effect.

1.9 "Agreement" has the meaning set forth in the Preamble.

1.10 "Alliance Manager" has the meaning set forth in Section 2.1.

1.11 "Annual Net Sales" means, on a Licensed Product-by-Licensed Product basis, the total, aggregate Net Sales of such Licensed Product in the Territory in a particular Calendar Year.

1.12 "Antitrust Filings" has the meaning set forth in Section 2.3.2.

1.13 "Arbitration Request" has the meaning set forth in Section 11.3.

1.14 "Available" and "Availability" means: (a) with respect to a given Additional Target, that such Additional Target: [**].

1.15 "Biosimilar Product" means, with respect to a particular Licensed Product in a particular country in the Territory: (a) any pharmaceutical or biological product sold by a Third Party that is not a Sublicensee of Novartis or its Affiliates and that did not purchase such product in a chain of distribution that included Novartis or any of its Affiliates or Sublicensees; and (b) which pharmaceutical or biological product (i) is approved by the applicable Regulatory Authority as biosimilar to, or interchangeable with, such Licensed Product (including, with respect to the United States, a product that is the subject of an application submitted under Section 351(k) of the Public Health Services Act citing the Licensed Product as the reference product), (ii) for which the Regulatory Approval otherwise references or relies on such Licensed Product as a reference product or any corresponding foreign application in the Territory (including, with respect to the EU, a marketing authorization application for a biosimilar biological medicinal product pursuant to Article 10(4) of Directive 2001/83/EC, or (iii) otherwise utilizes the same Capsid, in combination with the same Novartis Payload, or a payload substantially similar in structure and function to the Novartis Payload, and is directed to the Licensed Target utilized by such Licensed Product; provided that such pharmaceutical or biological product is not the subject of an enforcement action brought by Voyager in accordance with Section 6.3.3.

1.16 "BLA" means (a) an application requesting permission from the FDA to introduce, or deliver for introduction, a biopharmaceutical product into interstate commerce, or (b) any similar application or submission for Marketing Approval of a biopharmaceutical product filed with a Regulatory Authority in a country or group of countries.

1.17 "Business Day" means a day other than: (a) a Saturday or Sunday; (b) a holiday observed by the United States federal government or the Commonwealth of Massachusetts; or (c) a public holiday on which the banks are open for business in Basel, Switzerland.

1.18 "Calendar Quarter" means a period of three (3) consecutive months ending on the last day of March, June, September, or December, respectively; provided that: (a) the first Calendar Quarter during the Term will begin on the Effective Date and end on the last day of the Calendar Quarter within which the Effective Date falls; and (b) the last Calendar Quarter during the Term will end upon the effective date of expiration or termination.

1.19 "Calendar Year" means a period of twelve (12) consecutive months beginning on January 1 and ending on December 31; provided that: (a) the first Calendar Year starts on the Effective Date and ends on December 31, 2021; and (b) the last Calendar Year starts on January 1 of such year and ends on the effective date of expiration or termination.

1.20 "Campaign" means (a) completion of at least [**] rounds of screening of Capsid candidates in a campaign directed to identification of Capsids useful for Development of AAV Gene Therapy, excluding intermediate round screening results (other than such intermediate data that may be available and requested by Novartis within [**] before the expiration of the Research Term(s) or Additional Target Option Period), or (b) completion of subsequent rounds or screening associated with the evolution of Capsids of interest identified following the original multi-round screening specified in (a) above; excluding in each case ((a) and (b)) any such campaign conducted specifically for a Third Party.

1.21 "Capsid" means the protein shell of an AAV, consisting of oligomeric structural subunits made of certain proteins.

1.22 "Capsid Candidate" means any proprietary Capsid created by Voyager and made available to Novartis for Evaluation.

1.23 "Capsid Patent" means any Patent Controlled by Voyager as of the Effective Date or at any time during the Term with claims that Cover: (a) compositions of matter of any Capsid Candidate or Licensed Capsid; or (b) methods of use of any Capsid Candidate or Licensed Capsid; in each case (a) and (b), including any Patent Controlled by Voyager that contains a claim that Covers a Capsid Candidate or Licensed Capsid alone or in combination with any payload, including a Novartis Payload. The Capsid Patents existing as of the Effective Date are set forth in Schedule 1.22, which exhibit shall be updated annually by Voyager.

1.24 "Change of Control" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock redemption, cancellation, or other recapitalization, and immediately after such acquisition or increase such Third Party is, directly or indirectly, the beneficial owner of voting securities representing more than fifty percent (50%) of the total voting power of all of the then outstanding voting securities of such Party; (b) any merger, consolidation, recapitalization, or reorganization of such Party is consummated that would result in shareholders or equity holders of such Party immediately prior to such transaction owning less than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve any plan of complete liquidation of such Party, or an agreement for the sale or disposition by such Party of all or substantially all of such Party's assets, in each case, through one or more related transactions, other than to an Affiliate or pursuant to one or more related transactions that would result in shareholders or equity holders of such Party immediately prior to such transaction owning more than fifty percent (50%) of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; or (d) the sale or transfer to any Third Party, in one or more related transactions, of all or substantially all of such Party's consolidated assets taken as a whole.

1.25 "Clinical Trial" means a human clinical study conducted on sufficient numbers of human subjects that is designed to: (a) establish that a biopharmaceutical product is reasonably safe for continued human testing; (b) investigate the safety and efficacy of the biopharmaceutical product for its intended use, and to define warnings, precautions and adverse reactions that may be associated with the pharmaceutical product in the dosage range to be prescribed; or (c) support Regulatory Approval of a biopharmaceutical product or label expansion of a pharmaceutical product.

1.26 "Commercialization" means any and all activities directed to the marketing, promotion, distribution, offering for sale, sale, having sold, importing, having imported, exporting, having exported or other commercialization of a pharmaceutical or biologic product, but excluding

activities directed to Manufacturing or Development. "Commercialize", "Commercializing", and "Commercialized" have correlating meanings.

1.27 "Commercially Reasonable Efforts" means, with respect to the efforts to be expended by a Party with respect to any objective, those reasonable, good faith efforts to accomplish such objective as such Party would normally use to accomplish a similar objective under similar circumstances. With respect to any efforts relating to the Development, Regulatory Approval, or Commercialization of a Licensed Product by Novartis, generally or with respect to any particular country in the Territory, [**]. It is anticipated that the level of effort may change over time, reflecting changes in the status of a Licensed Product. Further, to the extent that the performance of a Party's obligations hereunder is adversely affected by the other Party's failure to perform its obligations hereunder, the impact of such performance failure will be taken into account in determining whether such Party has used its Commercially Reasonable Efforts to perform any such affected obligations.

1.28 "Competitive Infringement" means infringement of a Licensed Patent by a product that is competitive with a Licensed Product.

1.29 "Confidential Information" has the meaning set forth in Section 7.1.

1.30 "Control" means, with respect to a Person and any Know-How or Patent, the possession by such Person of the right (whether through ownership, license, or otherwise (other than by a license under this Agreement)) to grant the rights and licenses as provided herein, without violating the terms of any agreement with any Third Party. Notwithstanding the foregoing, in the event that a Third Party becomes an Affiliate or assignee of a Party after the Effective Date as a result of a Change of Control of such Party (such Third Party, together with its Affiliates immediately prior to the consummation of such Change of Control, the "Acquiring Entities"), the following will be deemed to be not Controlled by such Party or any of its Affiliates: (a) any Patent, Know-How, Regulatory Filing, or Regulatory Approval owned or otherwise controlled by such Acquiring Entity immediately prior to the consummation of such Change of Control; and (b) any Patent, Know-How, Regulatory Filing, or Regulatory Approval developed by or on behalf of such Acquiring Entity outside the scope of activities under this Agreement or acquired by or on behalf of such Acquiring Entity after the consummation of such Change of Control.

1.31 "Cover" means with regard to a particular subject matter and a Valid Claim in a Patent, that in the absence of ownership of or a license granted under such Valid Claim in such Patent, the making, use, offer for sale, sale, importation, Development, Manufacture, or Commercialization of such subject matter, would infringe such Valid Claim in such Patent.

1.32 "Debtor" has the meaning set forth in Section 10.6.1.

1.33 "Defense Proceeding" means an opposition, reexamination request, action for declaratory judgment, nullity action, interference or post-grant proceeding or other attack upon the validity, title or enforceability of a Patent that occurs in the context of litigation; excluding any such proceeding brought as a counterclaim to or defense of, or that accompanies a defense of, any enforcement action under Section 6.3.3.

1.34 "Development" means all internal and external research, development, and regulatory activities related to pharmaceutical or biologic products, including: (a) research, non-clinical testing, toxicology, testing and studies, non-clinical and preclinical activities, and Clinical Trials; and (b) preparation, submission, review, and development of data or information for the purpose of submission to a Regulatory Authority to obtain authorization to conduct Clinical Trials and to obtain, support, or maintain Regulatory Approval of a pharmaceutical or biologic product and interacting with Regulatory Authorities following receipt of Regulatory Approval in the applicable country or region for such pharmaceutical or biologic product regarding the foregoing, but excluding activities directed to Manufacturing or Commercialization. "Development" includes development and regulatory activities for additional forms, formulations, or indications for a pharmaceutical or biologic product after receipt of Regulatory Approval of such product (including label expansion), including Clinical Trials initiated following receipt of Regulatory Approval or any Clinical Trial to be conducted after receipt of Regulatory Approval that was mandated by the applicable Regulatory Authority as a condition of such Regulatory Approval with respect to an approved formulation or indication (such as post-marketing studies, observational studies, implementation and management of registries and analysis thereof, in each case, if required by any Regulatory Authority in any region in the Territory to support or maintain Regulatory Approval for a pharmaceutical or biologic product in such region). "Develop", "Developing", and "Developed" have correlating meanings.

1.35 "Development Milestone Event" means any Milestone Event set forth in Section 5.4.1.

1.36 "Development Milestone Event Notice" has the meaning set forth in Section 5.4.1.

1.37 "Development Milestone Payment" has the meaning set forth in Section 5.4.2.

1.38 "Diligence Issue" has the meaning set forth in Section 4.2.5.

1.39 "Disclosing Party" has the meaning set forth in Section 7.1.

1.40 "Dollars" or "$" means the legal tender of the U.S.

1.41 "Effective Date" has the meaning set forth in the Preamble.

1.42 "EMA" means the European Medicines Agency, and any successor entity thereto.

1.43 "Evaluate" means evaluation conducted by or on behalf of Novartis during the Research Term, to assess any Capsid Candidate and to determine its interest in exercising an Option or substitution right for such Capsid Candidate. "Evaluation" and "Evaluating" have correlating meanings.

1.44 "Evaluation Start Date" means: (a) with respect to the Initial Targets, the Effective Date; and (b) with respect to any Additional Target, the date upon which Novartis pays the Additional Target Option Fee in accordance with Section 5.2.

1.45 "Executive Officers" means: (a) with respect to Voyager, Voyager's Chief Executive Officer, or his or her designee; or (b) with respect to Novartis, [**], or his or her designee.

1.46 "Existing Confidentiality Agreement" has the meaning set forth in Section 7.1.4.

1.47 "Exploit" means to Develop, Manufacture, Commercialize, or otherwise exploit. "Exploitation" and "Exploiting" have correlating meanings.

1.48 "FDA" means the U.S. Food and Drug Administration, and any successor entity thereto.

1.49 "FD&C Act" means the U.S. Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

1.50 "First Commercial Sale" means, with respect to a Licensed Product, the first sale in an arms'-length transaction of such Licensed Product to a Third Party(excluding Sublicensees or distributors) of such Licensed Product in such country after all Regulatory Approvals (including Price Approvals) have been granted by the applicable Regulatory Authority of such country or, if Regulatory Approval is not required, after the date on which sales are permitted by applicable Law. For clarity, the First Commercial Sale of a Licensed Product shall not include any distribution or other sale at or below cost solely for patient assistance, named patient use, compassionate use, or test marketing programs or non-registrational studies or similar programs or studies where the Product is supplied without charge or at the actual manufacturing cost thereof.

1.51 "Functionally Equivalent Variant" means with respect to any Licensed Capsid, any Capsid derived by or on behalf of Voyager from the Licensed Capsid (including any modification thereof) that meets each of the following criteria as compared to the Licensed Capsid: [**].

[**].

1.52 "Global Trade Control Laws" has the meaning set forth in Section 11.8.

1.53 "Governmental Authority" means any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

1.54 "HSR Act" has the meaning set forth in Section 2.3.2.

1.55 "IND" means an investigational new drug application (including any amendment or supplement thereto) submitted to the FDA pursuant to U.S. 21 C.F.R. Part 312, including any amendments thereto, or any comparable filing(s) outside the United States for the investigation of any product in any other country or group of countries.

1.56 "Indemnified Party" has the meaning set forth in Section 9.3.

1.57 "Indemnifying Party" has the meaning set forth in Section 9.3.

1.58 "Indirect Taxes" has the meaning set forth in Section 5.13.2.

1.59 "Infringement Notice" has the meaning set forth in Section 6.3.1.

1.60 "Initial Targets" means the following genes, including the coding and non-coding regions affecting their function and regulation, and modifications thereof: *[**]*.

1.61 "Initiation" means, with respect to any Clinical Trial, first dosing of the first human subject in such Clinical Trial.

1.62 "Invention" or "Invented" means the result or act of invention (whether patentable or not) as determined in accordance with U.S. patent laws.

1.63 "Joint Inventions" has the meaning set forth in Section 6.1.3.

1.64 "Joint Patents" means all Patents within the Joint Inventions.

1.65 "Know-How" means all proprietary information, know-how and data, including trade secrets, Inventions (whether patentable or not), discoveries, methods, specifications, processes, procedures, formulas, expertise, technology, data (including non-clinical, pre-clinical and clinical data), documentation, materials, and results (including pharmacological, toxicological, biological, chemical, physical, safety and Manufacturing data and results), analytical and quality control data and results, Manufacturing techniques, Regulatory Filings and other technical information. "Know-How" excludes in any event any Patents.

1.66 "Law" means any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.67 "Licensed Capsid" means, with respect to a Licensed Target: (a)(i) a Capsid Candidate for which Novartis has exercised the applicable Option for such Licensed Target in accordance with Section 2.3.1 and paid the applicable Option Exercise Fee; or (ii) any Substitute Capsid that Novartis has designated as a replacement for a previously-designated Licensed Capsid in accordance with Section 2.4; (b) any Functionally Equivalent Variant of the Capsid Candidate or Substitute Capsid described in (a)(i) or (a)(ii) as applicable; or (c) any Capsid derived by or on behalf of Novartis or its Affiliates from a Capsid described in (a) or (b) above where such derived Capsid (i) is Covered by a Capsid Patent, (ii) contains changes or improvements, and (iii) such changes or improvements are not Covered by a claim of a Capsid Patent that is patentably distinct from the claims of the Capsid Patent that Cover the initial Capsid set forth in (a) or (b) above.

1.68 "Licensed Capsid Patent" means, collectively, any Capsid Patent that Covers any Licensed Capsid, but excluding any Licensed Product Patents.

1.69 "Licensed Field" means all indications for therapeutic, diagnostic and prophylactic human and veterinary use.

1.70 "Licensed Product" means a product comprising: (a) a Licensed Capsid; and (b) a Novartis Payload directed to a Licensed Target for which Novartis exercised its Option for such Licensed Capsid, as identified in Novartis's Option Exercise Notice.

1.71 "Licensed Product Patent" means, collectively, any Patent Controlled by Novartis at any time during the Term with claims directed to the combination of a Licensed Capsid and a Novartis Payload together or any method of use directed to such combination.

1.72 "Licensed Target" means any Subject Target for which an Option is exercised, following the exercise of the applicable Option and payment of the applicable Option Exercise Fee.

1.73 "Litigation Conditions" has the meaning set forth in Section 9.4.

1.74 "Losses" has the meaning set forth in Section 9.1.

1.75 "Major Market Country" means the United Kingdom, France, Germany, Italy, Spain and Japan.

1.76 "Manufacture" means activities directed to manufacturing, processing, packaging, labeling, filling, finishing, assembly, quality assurance, quality control, testing, and release, shipping, or storage of any pharmaceutical or biologic product (or any components or process steps involving any product or any companion diagnostic), placebo, or comparator agent, as the case may be, including process development, qualification, and validation, scale-up, pre-clinical, clinical, and commercial manufacture and analytic development, product characterization, and stability testing. "Manufacturing" has correlating meaning.

1.77 "Net Sales" means the net sales recorded by Novartis or any of its Affiliates or Sublicensees, excluding distributors and wholesalers, for any Licensed Product sold to Third Parties other than Sublicensees as determined in accordance with Novartis' Accounting Standards as consistently applied, less a deduction of [**] percent ([**]%) for direct expenses related to the sales of the Product, distribution and warehousing expenses and uncollectible amounts on previously sold products. The deductions booked on an accrual basis by Novartis and its Affiliates under its Accounting Standards to calculate the recorded net sales from gross sales include, without limitation, the following:

(a) normal trade and cash discounts;

(b) amounts repaid or credited by reasons of defects, rejections, recalls or returns;

(c) rebates and chargebacks to customers and third parties (including, without limitation, Medicare, Medicaid, Managed Healthcare and similar types of rebates);

(d) amounts provided or credited to customers through coupons and other discount programs;

(e) delayed ship order credits, discounts or payments related to the impact of price increases between purchase and shipping dates or retroactive price reductions;

(f) fee for service payments to customers for any non-separable services (including compensation for maintaining agreed inventory levels and providing information); and

(g) other reductions or specifically identifiable amounts deducted for reasons similar to those listed above in accordance with Novartis' Accounting Standards.

With respect to the calculation of Net Sales:

i. Net Sales only include the value charged or invoiced on the first arm's length sale to a Third Party;

ii. sales between or among Novartis and its Affiliates and Sublicensees shall be disregarded for purposes of calculating Net Sales; and

iii. If a Licensed Product is delivered to the Third Party before being invoiced (or is not invoiced), Net Sales will be calculated at the time the revenue recognition criteria under Novartis Accounting Standards are met.

1.78 "Non-Disclosing Party" has the meaning set forth in Section 7.5.

1.79 "Novartis" has the meaning set forth in the Preamble.

1.80 "Novartis Background IP" has the meaning set forth in Section 6.1.1.

1.81 "Novartis Evaluation Data" has the meaning set forth in Section 2.2.2.

1.82 "Novartis Payload" means a DNA sequence utilized by Novartis that is intended to have a therapeutic effect on a Subject Target when packaged into a Capsid and delivered to the appropriate cells.

1.83 "Option" has the meaning set forth in Section 2.3.1.

1.84 "Option Exercise Date" has the meaning set forth in Section 2.3.1.

1.85 "Option Exercise Fee" has the meaning set forth in Section 5.3.

1.86 "Option Exercise Notice" has the meaning set forth in Section 2.3.1.

1.87 "Other Know-How" has the meaning set forth in Section 2.3.1.

1.88 "Patent" means (a) any patent, patent application or utility models (including any provisional application, priority application, or international applications) in any country or multinational jurisdiction in the Territory (including any converted application, continuation, continuation-in-part, continued prosecution application or divisional of any such application, any reissue, renewal, extension, registration, confirmation, revalidation, restoration, substitution, reexamination, supplementary protection certificate, pediatric exclusivity period or the like of any such patent); (b) any foreign equivalent of any patent or patent application described in clause (a); and (c) all rights of priority in any of the foregoing.

1.89 "Parties" or "Party" has the meaning set forth in the Preamble.

1.90 "Person" means any individual, partnership, limited liability company, firm, corporation, association, trust, unincorporated organization or other similar entity or organization.

1.91 "Phase I Clinical Trial" means a Clinical Trial (or a portion of a human clinical trial) of a product in any country, the principal purpose of which is a preliminary determination of safety in healthy individuals or patients, that would satisfy the requirements of 21 C.F.R. 312.21(a), or a similar clinical study prescribed by the relevant Regulatory Authorities in a country other than the United States.

1.92 "Pivotal Clinical Trial" means a Clinical Trial of a Licensed Product that either (a) would satisfy the requirements of 21 C.F.R. 312.21(c) or corresponding foreign regulations; or (b) is intended (as of the time the Clinical Trial is Initiated) to obtain sufficient data to support the filing of a BLA for such Licensed Product. Pivotal Trial may include (i) a Clinical Trial that is designed to satisfy the requirements of both 21 C.F.R. 312.21(b) and 21 C.F.R. 312.21(c) or corresponding foreign regulations, or (ii) a Clinical Trial that is designed to satisfy the requirements of 21 C.F.R. 312.21(b) that is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. 312.21(c) or to provide sufficient data to support the filing of a BLA for such Licensed Product, as supported by a Regulatory Authority's formal meeting minutes or comparable documents, in which case such Pivotal Trial shall be deemed to have been Initiated upon the first dosing of the first human subject under the optimized or expanded protocol for such Clinical Trial.

1.93 "Price Approval" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of such reimbursement authorization or pricing approval or determination (as the case may be).

1.94 "Prosecution and Maintenance" or "Prosecute and Maintain" means, with regard to a Patent: (a) the preparation, filing, prosecution, maintenance, and requests for patent term adjustments or patent term extensions, including terminally disclaiming an application or issued patent of or for such Patent, as well all appeals therefrom; and (b) any proceeding, other than routine *ex parte* prosecution, which challenges such Patent occurring independently of litigation of the Patent, including re-examinations, nullity actions, interferences, oppositions, derivation

proceedings, post-grant reviews, reissues, and other similar proceedings with respect to such Patent and any appeals therefrom.

1.95 "Rare Disease Target" means a gene target (a) whose expression is intended to be replaced, increased, knocked down, or otherwise modulated and (b) that corresponds to a prevalent population of fewer than 200,000 patients in the United States.

1.96 "Receiving Party" has the meaning set forth in Section 7.1.

1.97 "Redacted Version" has the meaning set forth in Section 7.4.2.

1.98 "Regulatory Approval" means the approval of the applicable Regulatory Authority necessary for the marketing and sale of a product in a country(ies), including any required Price Approval.

1.99 "Regulatory Approval Application" means a Regulatory Filing submitted to an applicable Regulatory Authority to obtain Regulatory Approval to market and sell a particular product in the country or countries that such Regulatory Authority is responsible for, including any amendments thereto and supplemental applications.

1.100 "Regulatory Authority" means the FDA in the United States or any Governmental Authority in another country in the Territory that is a counterpart to the FDA and holds responsibility for granting Regulatory Approval for a product in such country, including the EMA, and any successor(s) thereto.

1.101 "Regulatory Filing" means, with respect to a product, any documentation comprising or relating to or supporting any filing or application with any Regulatory Authority with respect to such product, or its use or potential use in the Field, including any document submitted to any Regulatory Authority, including any IND, any Regulatory Approval Application and any correspondence with any Regulatory Authority with respect to such product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

1.102 "Relevant Capsid Patents" has the meaning set forth in Section 8.2.2.

1.103 "Relevant Factors" means all relevant factors that may affect the Development, Regulatory Approval or Commercialization of a Licensed Product, including (as applicable): actual and potential issues of safety, efficacy or stability; product profile (including product modality, category and mechanism of action); stage of development or life cycle status; actual and projected Development, Regulatory Approval, Manufacturing, and Commercialization costs; any issues regarding the ability to Manufacture or have Manufactured any Licensed Capsid or Licensed Product; the likelihood of obtaining Regulatory Approvals (including satisfactory or required Price Approvals); the timing of such approvals; the current guidance and requirements for Regulatory Approval for the Licensed Product and similar products and the current and projected regulatory status; labeling or anticipated labeling; the then-current competitive environment and the likely competitive environment at the time of projected entry into the market; past performance of the Licensed Product or similar products; present and future market potential; the ability to obtain adequate supply of any Licensed Capsid or Licensed Product, or any component thereof, from any

Third Party as may be required to Develop, secure Regulatory Approval for or Commercialize any Licensed Capsid or Licensed Product; Patent Rights of a Third Party; existing or projected pricing, sales, reimbursement and profitability; pricing or reimbursement changes in relevant countries; proprietary position, strength and duration of patent protection and anticipated exclusivity; and other relevant scientific, technical, operational and commercial factors.

1.104 "Representatives" means: (a) with respect to Novartis, Novartis and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents; and (b) with respect to Voyager, Voyager and its Affiliates and each of their respective officers, directors, employees, consultants, contractors, and agents.

1.105 "Research Term" means, on a Subject Target-by-Subject Target basis, the period commencing on the Evaluation Start Date for such Subject Target and ending on the first to occur of: (a) the first (1st) anniversary of the Evaluation Start Date; or (b) if the Option for such Subject Target is exercised, the third (3rd) anniversary of the Evaluation Start Date.

1.106 "Residual Knowledge" means knowledge, techniques, experience and Know-How that: (a) are, or are based on any Confidential Information Controlled by the Disclosing Party; and (b) are retained in the unaided memory of any authorized Representative of the Receiving Party after having access to such Confidential Information. An individual's memory will be considered to be unaided if the individual has not intentionally memorized the Confidential Information for the purpose of retaining and subsequently using or disclosing it. In no event, however, will Residual Knowledge include any knowledge, techniques, experience and Know-How to the extent (at any time, for such time) within the scope of any issued, valid, and enforceable patent claim Controlled by the Disclosing Party.

1.107 "Restricted Market" has the meaning set forth in Section 11.8.1.

1.108 "Restricted Party" has the meaning set forth in Section 11.8.2.

1.109 "Royalty Floor" has the meaning set forth in Section 5.7.4.

1.110 "Royalty Term" means, on a country-by-country and Licensed Product-by-Licensed Product basis, the period commencing on the First Commercial Sale of such Licensed Product in such country and terminating upon the latest to occur of: (a) expiration of the last Valid Claim of a Licensed Capsid Patent Covering the Licensed Product in such country; (b) termination or expiration of regulatory or data exclusivity for such Licensed Product in such country; and (c) [**] after the First Commercial Sale of such Licensed Product in such country.

1.111 "Sublicense" has the meaning set forth in Section 3.2.

1.112 "Sublicensee" has the meaning set forth in Section 3.2.

1.113 "Substitute Capsid" has the meaning set forth in Section 2.4.

1.114 "Subject Targets" means, collectively, the Initial Targets and Additional Targets. "Subject Target" means a Subject Target.

1.115 "Tax Action" has the meaning set forth in Section 5.13.3.

1.116 "Term" has the meaning set forth in Section 10.1.

1.117 "Territory" means worldwide.

1.118 "Third Party" means any Person that is neither a Party nor an Affiliate of a Party.

1.119 "Third Party Claims" has the meaning set forth in Section 9.1.

1.120 "Third Party License" has the meaning set forth in Section 5.7.2.

1.121 "United States" or "U.S." means the United States of America and all of its territories and possessions.

1.122 "Valid Claim" means, with respect to a particular country and Licensed Product: (a) a claim of an issued and unexpired Licensed Capsid Patent (i) that has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other Governmental Authority of competent jurisdiction from which no appeal can be taken or has not been appealed within the time allowed for appeal and (ii) that has not been irrevocably abandoned, disclaimed, denied, or admitted to be invalid or unenforceable through reissue, re-examination, or disclaimer or otherwise; or (b) a claim of a pending patent application within the Licensed Capsid Patent(s) that has not been cancelled, withdrawn, abandoned or finally rejected by an administrative agency action from which no appeal can be taken, provided that any claim in any patent application pending for more than [**] from the earliest date on which such claim claims priority shall not be considered a Valid Claim for purposes of the Agreement from and after such [**] date.

1.123 "Voyager" has the meaning set forth in the Preamble.

1.124 "Voyager Background IP" has the meaning set forth in Section 6.1.2.

1.125 "Voyager Know-How" means Know-How that: (a) is Controlled by Voyager or any of its Affiliates as of the Effective Date or that comes into the Control of Voyager or any of its Affiliates during the Term (other than through the grant of a license by Novartis); (b) is disclosed or is required to be disclosed by or on behalf of Voyager to Novartis in connection with this Agreement; and (c) relates to any Capsid Candidate or Licensed Capsid or the Exploitation of any Capsid Candidate or Licensed Capsid. "Voyager Know-How" expressly excludes any Know-How relating to Voyager's proprietary SF9 manufacturing technology.

1.126 "Voyager's Knowledge" means the actual knowledge, as of the Effective Date, of Voyager's [**].

ARTICLE 2
RESEARCH AND LICENSE OPTION

2.1 Alliance Managers. Within [**] after the Effective Date, each Party will appoint an individual to act as an alliance manager for such Party (each, an "Alliance Manager"). The Alliance

Managers will be the primary point of contact for the Parties under this Agreement, including with regard to Voyager's disclosure of any Capsid Candidates and Novartis's Evaluation of any Capsid Candidate. The name and contact information for each Party's Alliance Manager, as well as any replacement chosen by such Party, in its sole discretion, from time to time, will be promptly provided to the other Party in writing. Each Party may change its designated Alliance Manager at any time upon written notice to the other Party; provided that each Party will maintain an Alliance Manager throughout the duration of the Term. The Parties may mutually agree in writing to eliminate the requirement to maintain an Alliance Manager at any point following the expiration of the last to expire Research Term.

2.2 Capsid Candidate Evaluation.

2.2.1 Campaigns and Disclosure. During the Research Term, Voyager may (but will not be obligated to), at Voyager's sole discretion and expense, conduct Campaigns and identify Capsid Candidates that may be useful for AAV Gene Therapy. Voyager will disclose to Novartis, on a rolling basis (but no less frequently than [**]), the data relating to the performance characteristics for Capsids that may be useful for AAV Gene Therapy that (a) is or becomes Controlled by Voyager during the Research Term, or (b) arises from Campaigns conducted during the Research Term.

2.2.2 Evaluation of Capsid Candidates. During the Research Term for each Subject Target, following the disclosure by Voyager to Novartis of a Capsid Candidate, Novartis will have the right, in its sole discretion, to select [**] such Capsid Candidates for Evaluation by written notice to Voyager, and upon receipt of such written notice, Voyager will promptly provide to Novartis plasmids for the production of each such Capsid Candidate for such Evaluation. Novartis will promptly provide to Voyager all results of such Evaluation that are generated during any Research Term to the corresponding Capsid Candidate that are related to biodistribution, expression level, and toxicity ("Novartis Evaluation Data"); provided that Novartis, in its sole discretion, may choose to redact, mask, or not provide any information related to a Novartis Payload. Voyager will be free to use the corresponding Evaluation data for its own internal research purposes, in support of Voyager's Patent filings, and as part of data packages shared under confidentiality in association with the applicable Capsid Candidate (without attribution of the source of such data to Novartis); provided, however, that (a) Voyager shall not include Novartis Evaluation Data in any Patent filing without Novartis's prior written consent, which consent shall not be unreasonably withheld and (b) Voyager shall only share the Novartis Evaluation Data with Third Parties who have similarly contracted with Voyager to make available the results of such Third Party's evaluation of Capsids, subject to similar confidentiality protections. In the event Novartis does not exercise its Option for a particular Capsid Candidate, Novartis will not: (x) disclose the data from the corresponding Evaluation of such Capsid Candidate to any Third Party; or (y) include the data from the corresponding Evaluation of such Capsid Candidate in any Patent filing, except in each case of (x) or (y) where such data has become publicly available through no breach of this Agreement or with Voyager's prior written consent. Novartis may perform such Evaluation with respect to any Subject Target at any time during the applicable Research Term.

2.2.3 Additional Target Options.

(a) Additional Target Options. During the Additional Target Option Period, Novartis will have the option to nominate up to two (2) additional Rare Disease Targets for evaluation (each, an "Additional Target Option"). If Novartis desires to exercise an Additional Target Option, Novartis will provide Voyager with written notice identifying the proposed Rare Disease Target for which Novartis desires to exercise the Additional Target Option, including information regarding any applicable genetic variant(s) present in the intended addressable patient population sufficient for Voyager to determine whether the proposed target qualifies as a Rare Disease Target. Upon receipt of such notice, Voyager will provide written notice within [**] stating whether the proposed Rare Disease Target is Available at the time Voyager receives the above-described written notice from Novartis (and if Voyager reasonably disagrees that the proposed target qualifies as a Rare Disease Target).

(b) Additional Target Option Exercise. Within [**] of receipt of notice from Voyager that the proposed Rare Disease Target is Available (and qualifies as a Rare Disease Target) pursuant to Section 2.2.3(a), Novartis may exercise the Additional Target Option for such Rare Disease Target by providing written notice to Voyager of such exercise, and Novartis will pay the Additional Target Option Fee in accordance with Section 5.2.

(c) Non-Availability. If the proposed Rare Disease Target is not Available at the time Voyager receives the notice in Section 2.2.3(a), or it is determined that the proposed target does not qualify as a Rare Disease Target, Novartis will be deemed not to have exercised the Additional Target Option, and Novartis will continue to have the ability to nominate additional Rare Disease Targets for exercise of the Additional Target Option in accordance with the process set forth above in this Section 2.2.3 until its Additional Target Option has been exercised; provided that (i) if the time period for the Additional Target Option Period under Section 1.7 has expired following Novartis's nomination of the Rare Disease Target that was not Available, then (ii) Novartis's Additional Target Option will continue (and the time period for the Additional Target Option Period will be extended) for up to an additional [**] after Voyager's notification that the previously proposed Rare Disease Target is not Available (except if such period of [**] period is extended by mutual agreement of the Parties, repeating as necessary until a proposed Rare Disease Target is Available and Novartis triggers the Additional Target Option, or Novartis fails to timely nominate a Rare Disease Target; provided, however, that if for a given Additional Target Option no Rare Disease Target proposed by Novartis has been determined to be Available within [**] of the expiry of the Additional Target Option Period, then the Executive Officers shall meet to negotiate in good faith a methodology whereby a concurrence between the Rare Disease Targets of interest to Novartis and the Rare Disease Targets Available from Voyager may be determined. For the avoidance of doubt, the nomination process described in this Section 2.2.3 shall occur on a per Additional Target Option basis, and so the triggering of a one Additional Target Option does not foreclose the possibility of the process continuing for a second Additional Target Option.

2.2.4 Reporting. During the Research Term for each Subject Target, Voyager shall provide written reports summarizing the Capsid Candidates disclosed pursuant to Section 2.2.1, and Novartis will provide a written reports summarizing all results of the Evaluation conducted pursuant to Section 2.2.2 with timing to be mutually agreed by the Parties. The Alliance Managers will coordinate meetings to be held within [**] following receipt of such written reports

to discuss the contents of such reports, with each Party providing the appropriate personnel to address any reasonable inquiries of the other Party.

2.3 Option to License Capsid Candidates for Development and Commercialization of Licensed Products.

2.3.1 Voyager hereby grants to Novartis an option to receive the license as set forth in Section 3.1.2 for one (1) Capsid Candidate for each Subject Target (each, an "Option"). Novartis may exercise each Option during the applicable Research Term by providing written notice to Voyager, in accordance with Section 11.7, identifying the specific Capsid Candidate and Subject Target for which the Option is exercised (an "Option Exercise Notice"). Novartis may exercise each of its Options on the same or different Capsid Candidates for each Subject Target during the applicable Research Term but may only exercise its Option on one Capsid Candidate for each Subject Target (subject to the substitution rights as set forth in Section 2.4). Upon Voyager's receipt of each Option Exercise Notice (the "Option Exercise Date") each Capsid Candidate identified in the corresponding Option Exercise Notice will be deemed a "Licensed Capsid" for the selected Subject Target, which will be deemed a "Licensed Target." Promptly following receipt of Novartis's Option Exercise Notice, Voyager will issue the appropriate invoice in accordance with Section 5.4.2 and provide Novartis with any Voyager Know-How for the corresponding Licensed Capsid that has not been previously provided to Novartis as may be reasonably necessary or that the Parties mutually agree may be useful to enable Novartis to Exploit such Licensed Capsid for use in Licensed Products; provided that Voyager shall not provide Novartis with any Know-How that is not reasonably necessary for Exploiting a Licensed Capsid (such Know-How, "Other Know-How") without Novartis' prior written consent. In the event Voyager provides Other Know-How without Novartis' prior written consent, then Novartis shall have the right to use such Other Know-How for Development of Licensed Products, provided that Voyager may notify Novartis of any inadvertent disclosure of Other Know-How and Novartis shall destroy all such Other Know-How to the extent not previously relied upon in the Development of Licensed Products.

2.3.2 All Option exercise notices delivered by Novartis shall specify whether the exercise of the applicable Option, in Novartis's good faith assessment based on advice from specialized counsel, requires filings under the Hart-Scott-Rodino Antitrust Improvement Act (as amended from time to time, the "HSR Act") or similar antitrust or competition laws of other jurisdictions (collectively, the "Antitrust Filings"). If Novartis concludes that Antitrust Filings are required, then: (a) the Parties will (i) use reasonable efforts to make the requisite filings as promptly as possible, and in the case of filings under the HSR Act in any event no later than [**] after the exercise notice for the applicable Option, and (ii) collaborate with each other in taking appropriate steps to achieve expiration or termination of all applicable waiting periods as promptly as possible; and (b) the effectiveness of the relevant license(s) set forth in Section 3.1.2 shall be conditioned upon expiration or termination of such applicable waiting periods.

2.4 Capsid Substitution. After Option exercise but during the Research Term for any Subject Target, Novartis may conduct additional Evaluation of the Capsid Candidates for use with such Subject Target, and may elect to substitute any Capsid Candidate for any Licensed Capsid for which an Option has been exercised for such Subject Target by providing written notice to

Voyager, in accordance with Section 11.7, identifying the Capsid Candidate (the "Substitute Capsid") and the specific Licensed Target for which the Substitute Capsid will replace the previously designated Licensed Capsid. Immediately following Novartis's exercise of such substitution notice, the Substitute Capsid will replace the previous Licensed Capsid for such Licensed Target, and Voyager will provide Novartis with any Voyager Know-How for the Substitute Capsid that has not been previously provided to Novartis as may be reasonably necessary or that the Parties mutually agree may be useful to enable Novartis to Exploit such Substitute Capsid for use in Licensed Products directed to such Licensed Target.

2.5 Functionally Equivalent Variant Designation Based on [**]. Notwithstanding the criterion in Section 1.51(b), if during the Research Term, Novartis demonstrates and discloses to Voyager in writing with the relevant data (supporting or contrary) that [**], the criterion in Section 1.51(b) shall be deemed met with respect to the Capsid in question. Following the expiration of the Research Term, if Novartis demonstrates and discloses to Voyager in writing with the relevant data (supporting or contrary data) that [**], the criterion in Section 1.51(b) shall be deemed met with respect to the Capsid in question if, and only if, such Capsid is Available, as determined by Voyager. Voyager shall notify Novartis of such determination within [**] of receipt of all supporting data.

ARTICLE 3
GRANT OF LICENSES

3.1 Licenses to Novartis.

3.1.1 Research License. Subject to the terms and conditions of this Agreement, with respect to each Capsid Candidate, Voyager hereby grants to Novartis and its Affiliates , and Novartis hereby accepts, a non-exclusive (subject to Section 8.4.1), non-transferable (except in accordance with Section 11.4), non-sublicensable (except in the case of contractors performing services related to Evaluation for or on behalf of Novartis), worldwide, royalty-free right and license during the Research Term, under the Capsid Patents and Voyager Know How, to evaluate each Capsid Candidate for use with the applicable Subject Targets, in each case solely for the purpose of performing the Evaluation.

3.1.2 Exclusive Licenses from Voyager to Novartis.

(a) On a Licensed Capsid-by-Licensed Capsid basis and effective as of the Option Exercise Date for such Licensed Capsid, Voyager hereby grants to Novartis and its Affiliates an exclusive license (exclusive even as to Voyager) under the Licensed Capsid Patents and Voyager's interest in the Joint Patents, to use, have used, Develop, have Developed, Commercialize, and have Commercialized the applicable Licensed Capsid(s) as incorporated into Licensed Products containing the corresponding Novartis Payload directed to a Subject Target in the Territory.

(b) In the event Novartis assigns any Licensed Product Patent to Voyager in accordance with Section 6.2.3(b), Voyager hereby grants to Novartis and its Affiliates an exclusive (even as to Voyager), perpetual, non-revocable, world-wide, sub-licensable license

under such assigned Licensed Product Patent for all purposes, including Exploitation of any Capsid. The foregoing license shall be royalty free, except with respect to any granted and valid independent claim of such assigned Licensed Product Patent that recites the Licensed Capsid sequence or that otherwise relies upon recitation of the Licensed Capsid sequence for the novelty or non-obviousness of the claim (other than those Licensed Product Patents assigned under Section 6.2.3(b)(ii)), in which case Novartis shall be responsible for all payment obligations as would apply to a Licensed Capsid Patent.

Notwithstanding anything to the contrary, the exclusive licenses in this Section 3.1.2 are exclusive solely as each relates to the Exploitation of a Licensed Product in relation to a Subject Target.

3.1.3 Non-Exclusive License from Voyager to Novartis. Without limiting any other license granted under this Agreement, on a Licensed Capsid-by-Licensed Capsid basis and effective as of the Option Exercise Date, Voyager hereby grants to Novartis and its Affiliates a non-exclusive right and license, under the Voyager Know-How, to use, have used, Develop, have Developed, Manufacture, have Manufactured, Commercialize, and have Commercialized the applicable Licensed Capsid(s) as incorporated into Licensed Products containing a Novartis Payload directed to a Licensed Target in the Territory. The Parties acknowledge that Voyager Know-How expressly excludes any Know-How relating to Voyager's proprietary SF9 manufacturing technology. In the event Novartis wishes to consult Voyager on manufacturing Know-How of the Capsids, Voyager agrees to cooperate with Novartis and the Parties will modify the Agreement as appropriate.

3.1.4 Right of Reference. Voyager hereby grants to Novartis a "Right of Reference," as that term is defined in 21 C.F.R. § 314.3(b) (or any analogous Law recognized outside of the United States), to all data Controlled by Voyager or its Affiliates that relates to any Licensed Capsid or Licensed Product solely for purposes of seeking Regulatory Approval for Licensed Products, and Voyager shall provide a signed statement to this effect, if requested by Novartis, in accordance with 21 C.F.R. § 314.50(g)(3) (or any analogous Law outside of the United States).

3.2 Novartis's Sublicensing Rights. Novartis and its Affiliates will have the right to grant and authorize sublicenses through multiple tiers under the rights granted to it under this Agreement by Voyager, including Section 3.1.2 and Section 3.1.3 for the Exploitation of a Licensed Product (each such Third Party, a "Sublicensee"). Novartis will use Commercially Reasonable Efforts to include in each Sublicense, an obligation of the Sublicensee to provide Novartis with written notice of its achievement of a Development Milestone Event within [**] after such Sublicensee achieves the Development Milestone Event. Within [**] following execution of a sublicense with a Sublicensee (a "Sublicense"), Novartis will provide Voyager with a fully executed copy of the corresponding Sublicense, which copy may be redacted by Novartis to remove confidential or commercially sensitive information and any other information that is not necessary to demonstrate compliance with the terms of this Agreement. Each sublicense will be consistent with the terms of this Agreement. During the Term, Novartis will be responsible for any act or omission by a Sublicensee that would be a breach of this Agreement if such act or omission had been engaged in by Novartis. Novartis shall remain responsible for the payment to Voyager of all Development Milestone Payments, Sales Milestone Payments, and royalties that are payable

with respect to the Development Milestone Event(s) achieved by, or the Net Sales of, a Licensed Product made by such Sublicensees.

3.3 Voyager Rights.

3.3.1 Notwithstanding anything to the contrary set forth in this Agreement: (a) the exclusive licenses and exclusivity covenants set forth in this Agreement will not prevent Voyager from internal Development activities relating to the Capsid Candidates or Licensed Capsids, including any Development activities that may result in generation of Functionally Equivalent Variants; (b) nothing in this Agreement will prevent Voyager from Exploiting (or granting rights to an Affiliate or Third Party to Exploit) (i) subject to Section 8.4.1**,** any Capsid that is not a Licensed Capsid or a Functionally Equivalent Variant of such Licensed Capsid for use in connection with the Licensed Targets or (ii) any Licensed Capsid or Functionally Equivalent Variant of such Licensed Capsid for use with targets other than Licensed Target.

3.4 No Other Rights. Except as otherwise expressly provided in this Agreement, under no circumstances will a Party, as a result of this Agreement, obtain any ownership interest, license right or other right in any Know-How, Patent, or other intellectual property rights of the other Party or any of its Affiliates, including items owned, controlled, developed, or acquired by the other Party or any of its Affiliates, or provided by the other Party to the first Party at any time pursuant to this Agreement.

ARTICLE 4
DEVELOPMENT, REGULATORY AND COMMERCIALIZATION ACTIVITIES

4.1 Novartis Authority and Obligations.

4.1.1 As of each applicable Option Exercise Date, Novartis will be solely responsible for, and have sole decision-making authority with respect to, at its own expense, the Exploitation of Licensed Products and Licensed Capsids as they are used to Exploit a Licensed Product. During the Term, Voyager will be responsible for maintaining any Third Party agreement it has entered as of the Effective Date (if any) that is required for Novartis to practice the rights granted by Voyager to Novartis in the Agreement, including payment by Voyager of any amounts due under such Third Party agreements.

4.2 Diligence.

4.2.1 Development Diligence. Novartis will use Commercially Reasonable Efforts to Develop and obtain Regulatory Approval for at least one (1) Licensed Product for each Licensed Target in the United States and at least three (3) of the Major Market Countries. Novartis will have no other diligence obligations with respect to the Development or Regulatory Approval of Products under this Agreement.

4.2.2 Commercial Diligence. Novartis will use its Commercially Reasonable Efforts to Commercialize each Licensed Product in the United States and at least three (3) Major Market Countries in the Territory where Novartis or its designated Affiliates or Sublicensee has

received Regulatory Approval for such Licensed Product. Novartis will have no other diligence obligations with respect to the Commercialization of Products under this Agreement.

4.2.3 Exceptions to Diligence Obligations. Notwithstanding any provision of this Agreement to the contrary, Novartis will be relieved of its diligence obligations under this Agreement with respect to any Licensed Product to the extent that any of the following occurs with respect to such Licensed Product:

(a) Novartis or Voyager receives, generates, or otherwise becomes aware of, any safety, tolerability, or other data reasonably indicating or signaling that a Licensed Capsid or Licensed Product has or would have an unacceptable risk-benefit profile or is otherwise not reasonably suitable for initiation or continuation of Clinical Trials; or

(b) Novartis or Voyager receive any notice, information or correspondence from any applicable Regulatory Authority, or any applicable Regulatory Authority takes any action, that reasonably indicates that a Licensed Product is unlikely to receive Regulatory Approval.

4.2.4 Deemed Satisfaction of Novartis's Diligence Obligations. Without in any way expanding Novartis's obligations under this Agreement:

(a) Novartis's achievement of any Development Milestone Event entitling Voyager to receive a specific Development Milestone Payment described in Section 5.4.2 will be conclusive evidence that Novartis has satisfied all of its diligence obligations under this Agreement for the corresponding Licensed Product, up to the point such Development Milestone Event is achieved; and

(b) Novartis's payment, and Voyager's acceptance, of any Sales Milestone Payment as set forth in Section 5.4.3 will be conclusive evidence that Novartis has satisfied all its diligence obligations under this Agreement for the corresponding Licensed Product up to the date of the achievement of such milestone; provided that if Voyager does not return in full a Sales Milestone Payment by Novartis with a written rejection of such payment within [**] of receipt, Voyager shall be deemed to have accepted such Sales Milestone payment.

(c) For the avoidance of doubt, the provisions of Section 4.2.4 are intended only as examples of diligence constituting satisfaction of Novartis's diligence obligations. Novartis may fully satisfy its diligence obligations without achieving any of the specific diligence examples set forth in Section 4.2.4, *provided that* Novartis otherwise complies with the provisions of Section 4.2.1 or Section 4.2.2, as applicable.

4.2.5 Assertion of Novartis Diligence Obligation Claims. If Voyager is, becomes, or reasonably should be aware of facts that might form a reasonable basis to allege that Novartis has failed to meet any of its diligence obligations, then Voyager will promptly notify Novartis in writing of such potential alleged performance failure (each such potential alleged performance failure, a "Diligence Issue"). Promptly upon Novartis's receipt of any notice of a Diligence Issue pursuant to this Section 4.2.5, the Novartis Alliance Manager will contact the Voyager Alliance

Manager to discuss the specific nature of such Diligence Issue and seek to identify an appropriate corrective course of action. If, no later than [**] after Novartis's receipt of such a notice, (i) the Parties have not reached consensus regarding whether Novartis has failed to satisfy its obligations pursuant to Section 4.2.1 or Section 4.2.2 and (ii) the Parties' respective Alliance Managers have not agreed upon an appropriate corrective course of action for such Diligence Issue, then such Diligence Issue will be escalated and resolved pursuant to the dispute resolution provisions set forth in Section 11.2. If Voyager fails to notify Novartis of a Diligence Issue pursuant to this Section 4.2.5 within [**] after the date that Voyager first discovers or reasonably should have discovered such Diligence Issue, then Novartis will be deemed to have satisfied its obligations under Section 4.2.1 and Section 4.2.2 with respect to such Diligence Issue.

4.2.6 Remedies for Breach of Novartis Diligence Obligations. If Novartis materially breaches any of its diligence obligations under Section 4.2.1, and Novartis fails to timely remedy such breach within [**] after Novartis's receipt of notice of such breach from Voyager, or if such breach is not reasonably curable within [**] and if Novartis is making a *bona fide* effort to cure such breach, within a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach, the time to remedy such breach shall be extended for a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach), then Voyager may, in its sole discretion, elect to either: (i) terminate this Agreement pursuant to the provisions of Section 10.3.1 on a Licensed Product-by-Licensed Product and country-by-country basis; or (ii) convert any exclusive license or sublicense granted to Novartis under this Agreement into a non-exclusive license, solely in each case of (i) and (ii) with respect to a Licensed Product in the country that is the subject of the material breach;.

4.2.7 Reporting. Following the exercise of each Option and prior to the First Commercial Sale of the corresponding Licensed Product in each of the jurisdictions where any of the milestone payments under Section 5.4 remain outstanding, for each Licensed Product, Novartis will provide to Voyager a confidential [**] written report summarizing the material Development, Manufacture and Commercialization activities it has undertaken in such jurisdiction(s) during the preceding [**] period and the material Development, Manufacture and Commercialization activities it expects to take in the following [**] period, including any milestones expected to be achieved.

4.2.8 Cooperation. Upon Novartis's request and at Novartis's expense, Voyager will provide Novartis with reasonable assistance in connection with Novartis's preparation of any portion(s) of the relevant Regulatory Filings that relate to the Licensed Products, including by providing relevant data in Voyager's possession and participating in meetings between the Parties to prepare documents to be filed.

4.3 Remedy for Novartis Deprioritizing a Licensed Capsid. Without limiting Novartis's obligations under Section 4.2.1, on a Licensed Product-by-Licensed Product basis, if, during the period beginning on the corresponding Option Exercise Date for a Novartis Payload and ending on the date Novartis first Commercializes the corresponding Licensed Product in the United States and a Major Market Country, (i) Novartis declares a lead candidate incorporating a Novartis Payload for Development, and (ii) Novartis does not include a lead candidate or a back-up candidate incorporating a Licensed Capsid and a Novartis Payload in its Development efforts for

any contiguous [**] period (including efforts aimed at continuing Development through a Sublicensee), as indicated in Novartis's [**] report made under Section 4.2.7 and provided that such lack of inclusion is not a result of a matter set forth in Section 4.2.4(b), then, within [**] after receiving such report, Voyager shall notify Novartis in accordance with Section 11.7 of any objection it has to such lack of inclusion. If Voyager timely notifies Novartis of its objection and Novartis does not include a lead candidate or back-up candidate that incorporates the corresponding Licensed Candidate and Novartis Payload within [**] after Novartis's receipt of such notice, then Novartis may, in its sole discretion, immediately elect to convert the corresponding exclusive license or sublicense granted to Novartis under this Agreement into a non-exclusive license, with (a) all subsequent development obligations under Section 4.2.1 terminating for the corresponding Licensed Product and (b) all amounts for the corresponding Licensed Product that would be due hereunder after Voyager elects such non-exclusive license being reduced by [**] percent ([**]%).

4.4 Compliance. All activities to be conducted by a Party under this Agreement will be conducted in compliance with applicable Laws.

ARTICLE 5
INITIAL FEE; MILESTONES AND ROYALTIES; PAYMENTS

5.1 Upfront Fee. Novartis will pay Voyager an initial, one-time, non-refundable, non-creditable payment of Fifty-Four Million Dollars ($54,000,000) within [**] after the Effective Date.

5.2 Additional Target Option Fee. For each Additional Target Option that Novartis exercises, Novartis will pay to Voyager a fee of Eighteen Million Dollars ($18,000,000) per Additional Target (each, an "Additional Target Option Fee") following receipt of an invoice from Voyager in accordance with Section 5.5.

5.3 Option Exercise Fees. For each Option that Novartis exercises, Novartis will pay to Voyager a fee of Twelve Million Five Hundred Thousand Dollars ($12,500,000) (the "Option Exercise Fee") following receipt of an invoice from Voyager in accordance with Section 5.5.

5.4 Milestone Payments.

5.4.1 Generally. Novartis will provide Voyager with written notice (a "Development Milestone Event Notice") of the achievement of a development milestone event specified in Section 5.4.2 for the first Licensed Product, per Licensed Target, to achieve such milestone event (each, a "Development Milestone Event"). Such notice will be provided within [**] after such Development Milestone Event is achieved; provided that in the case such Development Milestone Event is achieved by a Sublicensee, Novartis's notice shall be provided within [**] after Novartis receives notice from the corresponding Sublicensee of achieving the Development Milestone Event.

5.4.2 Development Milestone Events and Payments. Notwithstanding anything to the contrary in this Agreement, this Section 5.4.2 shall apply only if Novartis exercises the

corresponding Option. Novartis will pay the milestones payments set forth below for the first Licensed Product for each Licensed Target to achieve such Development Milestone Event (each, a "Development Milestone Payment"). After receipt of a Development Milestone Event Notice, Voyager shall submit an invoice to Novartis substantially in the form of Exhibit A with respect to the corresponding Development Milestone Payment. If for any reason a Development Milestone Event (a) below does not occur prior to the occurrence of Development Milestone Event (b) below, then Development Milestone Event (a) will be deemed to occur concurrently with the occurrence of Development Milestone Event (b), and the Development Milestone Payments associated with both Development Milestone Events will be paid following the achievement of Development Milestone Event (b).

	Development Milestone Event	**Development Milestone Payment (per Licensed Target)**
(a)	[**]	[**] Dollars ($[**])
(b)	[**]	[**] Dollars ($[**])
(c)	[**]	[**] Dollars ($[**])
(d)	[**]	[**] Dollars ($[**])
(e)	[**]	[**] Dollars ($[**])
	Total Per Licensed Target	One Hundred Twenty-Five Million ($125,000,000)

The Development Milestones in (d) and (e) above will be deemed to have been achieved upon the occurrence of [**]. Each of the Development Milestone Payments set forth above will be payable one time only per Licensed Target incorporated into a Licensed Product (regardless of the number of Licensed Products with the same Licensed Target, or the number of times with respect to any Licensed Product with the same Licensed Target, achieves the specified Development Event occurs). No Development Milestone Payments will be payable by Novartis for any subsequent Licensed Product for a Licensed Target regardless of the number of Licensed Products for such Licensed Target are Developed. For clarification, if one Licensed Product replaces another Licensed Product in Development, then such replacement Licensed Product will only be subject to Development Milestone Payments that have not previously been triggered by one or more prior Licensed Products for the corresponding Licensed Target.

5.4.3 Sales Milestones. On a Licensed Product-by-Licensed Product basis, Novartis will pay to Voyager sales milestones with respect to Annual Net Sales of each Licensed Product for the first occurrence of each milestone event as follows:

	Milestone Event (per Licensed Product)	Sales Milestone Payment
(a)	First Calendar Year with Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])
(b)	First Calendar Year with Annual Net Sales exceeding of [**] Dollars ($[**])	[**] Dollars ($[**])
(c)	First Calendar Year with Annual Net Sales exceeding [**] Dollars ($[**])	[**] Dollars ($[**])
(d)	**Total Per Licensed Product**	One Hundred Seventy-Five Million Dollars ($175,000,000)

Upon receipt of a royalty report under Section 5.8 indicating that a sales milestone has been achieved, Voyager will provide an invoice for the applicable sales milestone. Upon receipt of an invoice, Novartis will pay Voyager the foregoing sales milestones in accordance with Section 5.5.

5.5 Invoicing and Payment Procedure. Voyager shall provide Novartis an invoice for all amounts due to it under this Agreement. Unless otherwise noted, all fees owed to Voyager will be payable within [**] after Novartis's receipt of an invoice from Voyager. All invoices will be delivered to Novartis by email to [**]. All invoice or billing related questions should be referred to Novartis's finance department at [**]. Invoices to Novartis shall be substantially in the form set forth in the Exhibit A. All payments due from Novartis to Voyager pursuant to this Agreement shall be made in U.S. dollars by wire transfer to the following bank account of Voyager (subject to confirmation) or to another bank account of Voyager specified in writing to Novartis and, in each case, in accordance with such instructions as are provided by Voyager to Novartis from time to time:

Account Name:	Voyager Therapeutics, Inc.
Bank:	[**]
Account Type:	[**]
Account Number:	[**]
Routing Number:	[**]
SWIFT Code:	[**]

IBAN:	[**]

5.6 Royalties.

5.6.1 Royalties on Licensed Products Sold.

(a) Annual Net Sales. Subject to the adjustments under Section 5.7, Novartis will make tiered royalty payments to Voyager in respect of Annual Net Sales, on a Licensed Product-by-Licensed Product basis, by Novartis, its Affiliates or Sublicensees at the following royalty rates during the applicable Royalty Term:

	Annual Net Sales of each Licensed Product in the Territory during a Calendar Year during the Royalty Term	**Royalty Rate**
(a)	Annual Net Sales less than [**] Dollars ($[**])	[**]%
(b)	Annual Net Sales greater than [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**]%
(c)	Annual Net Sales greater than [**] Dollars ($[**])	[**]%

5.6.2 Calculation of Royalties. Royalties on aggregate Annual Net Sales of each Licensed Products in a Calendar Year during the Royalty Term will be paid at the rate applicable to the portion of Net Sales within each of the Annual Net Sales tiers during such Calendar Year. For example, if, during a Calendar Year during the Royalty Term, Annual Net Sales of a Licensed Product are equal to $[**], then the royalties payable by Novartis would be calculated by [**].

5.7 Royalty Adjustments.

5.7.1 Valid Claim Expiration. If, during any Calendar Quarter during the Royalty Term, on a country-by-country and Licensed Product-by-Licensed Product basis, there is no Valid Claim within the Licensed Capsid Patents that Covers such Licensed Product in such country, then the royalty rate for such Licensed Product in such country will be reduced by [**] percent ([**]%) from the average rate(s) otherwise applicable as set forth in Section 5.6.1.

5.7.2 Third Party Licenses. In the event that, during the Royalty Term on a Licensed Product-by-Licensed Product basis, Novartis, its Affiliates or Sublicensees are required to pay royalties to a Third Party in consideration for a license under Patents Controlled by such Third Party that are reasonably necessary for Exploiting a Licensed Capsid as part of a Licensed Product in such country ("Third Party License "), then Novartis may deduct up to [**] percent ([**]%) of the royalties payable to such Third Party for such Third Party License(s) from royalties owed by Novartis to Voyager under Section 5.6.1 for Net Sales of the applicable Licensed Product with such reduction continuing until all such amounts have been expended for such Calendar Quarter; provided that royalties to a Third Party in consideration for a license under Patents

Controlled by a Third Party that are reasonably necessary for Exploiting the Novartis Payload or other component of a Licensed Product shall not be deductible against the royalties owed by Novartis to Voyager hereunder. In cases where royalties under the Third Party License are not readily attributable to a country, Novartis may allocate such royalties to countries using a reasonable methodology.

5.7.3 Biosimilar Products. If (a) for any Calendar Year in the applicable Royalty Term for a Licensed Product in a country in the Territory where (i) at least one (1) Biosimilar Product with respect to such Licensed Product is being sold in such country, and (ii) the Net Sales of such Licensed Product sold in such country in such Calendar Year are less than [**] percent ([**]%) as compared with the Net Sales of such Licensed Product in that country in the Calendar Year preceding the marketing or sale of the first Biosimilar Product, then (b), subject to Section 5.7.4, the royalty rate payable on Net Sales of such Licensed Product in such country in such Calendar Year would be reduced by [**] percent ([**]%) of the amounts of royalties otherwise applicable on such Net Sales pursuant to Section 5.6.1 for the remainder of the applicable Royalty Term, such reduction to be prorated appropriately in aggregate for the then-current Calendar Year.

5.7.4 Limit on Deductions. On a Licensed Product-by-Licensed Product basis, in no event will the cumulative effect of the adjustments in Sections 5.7.1 through Section 5.7.3 reduce the royalties payable to Voyager under Section 5.6.1 by more than [**] percent ([**]%) of the amounts that would otherwise have been payable with respect to the applicable Licensed Product in the applicable country in the applicable Calendar Quarter (the "Royalty Floor"). In the event that a reduction would be permitted under this Section 5.7 but for the fact that such reduction would reduce the applicable royalties payable in accordance with Section 5.6.1 by more than the Royalty Floor, then Novartis may carry over such royalty reduction to payments payable hereunder with respect to any royalty payments owed in any future Calendar Quarter, in each case with such reduction continuing until all such amounts have been expended.

5.7.5 Example. Exhibit B sets forth an example of the application of the royalty calculations set forth in this Section 5.7.

5.8 Reports; Payment of Royalty. During the Royalty Term, Novartis will furnish to Voyager a written report within [**] after the end of each Calendar Quarter showing, on a Licensed Product-by-Licensed Product and country-by-country basis, the Net Sales of each Licensed Product in each country of the Territory and the royalties payable under this Agreement. Upon receipt of a royalty report under this Section 5.8, Voyager will provide an invoice for the applicable royalty payments. Novartis will pay Voyager the foregoing royalties in accordance with Section 5.5.

5.9 Accounting; Audit.

5.9.1 Records. Novartis agrees to keep, and to require its Affiliates and Sublicensees to keep, full, clear and accurate records for a minimum period of [**] after the end of the calendar year to which they pertain, setting forth as applicable the Net Sales, in sufficient detail to enable royalties and compensation payable to Voyager hereunder to be determined.

5.9.2 Audits. Novartis agrees, upon not less than [**] prior written notice, to permit, and to require its Affiliates to permit, such books and records relating to such Licensed Products to be examined during regular business hours at such place or places where such records are customarily kept by an independent internationally-recognized accounting firm selected by Voyager and reasonably acceptable to Novartis for the purpose of verifying reports provided (or required to be provided) by Novartis under this Article 5. Any such audit will not be performed more frequently than [**] and not more frequently than [**] with respect to records covering any specific period of time and will be conducted under appropriate confidentiality provisions, for the sole purpose of verifying the accuracy and completeness of all financial, accounting and numerical information and calculations provided under this Agreement. In addition, Voyager shall only be entitled to audit the books and records of Novartis from the [**] prior to the calendar year in which the audit request is made. Before beginning its audit, the Auditor shall execute an agreement reasonably acceptable to the audited Party pursuant to which the Auditor agrees to keep confidential all information reviewed during the audit. The independent accounting firm will only share the results of the audit, not the underlying records, with the auditing party. Voyager agrees to treat as Novartis' Confidential Information all information received and all information learned in the course of any audit or inspection, except to the extent necessary to enforce its rights under this Agreement or to the extent required to comply with any law, regulation or judicial order.

5.9.3 Audit Reports and Disputes. The independent accounting firm will provide its audit report and the basis for any determination to Novartis at the time such report is provided to Voyager before such report is considered to be final. Novartis will have the right to request a further determination by such accounting firm as to matters which Novartis disputes within [**] following Novartis's receipt of such report. Novartis will provide Voyager and the accounting firm with a reasonably detailed statement of the grounds upon which it disputes any findings in the audit report and the accounting firm will undertake to complete such further determination, at Novartis's expense, within [**] after the dispute notice is provided, which determination will be limited to the disputed matters. Any matter that remains unresolved shall be resolved in accordance with the dispute resolution procedures contained in Section 11.2. In the event that the final result of the inspection reveals an undisputed underpayment or overpayment by Novartis, the underpaid or overpaid amount shall be settled promptly.

5.9.4 Audit Expenses. Except as provided in Section 5.9.3, any audit conducted by Voyager is to be made at the expense of Voyager, except if the results of the audit reveal an underpayment of royalties, milestones or other payments to Voyager under this Agreement of [**] percent ([**]%) or more in the audit period, in which case (a) Novartis will promptly remit to Voyager the amount of such underpayment and (b) the reasonable fees and expenses for such audit will be paid by Novartis.

5.10 Currency Conversion. Notwithstanding anything to the contrary in the Agreement, conversion of sales recorded in local currencies to U.S. dollars will be performed in a manner consistent with Novartis's normal practices used to prepare its audited financial statements for external reporting purposes, provided that such practices use a widely accepted source of published exchange rates.

5.11 Books and Records. Any books and records to be maintained under this Agreement by a Party or its Affiliates or Sublicensees will be maintained in accordance with Accounting Standards.

5.12 Methods of Payments. All payments due from Novartis to Voyager under this Agreement will be paid in Dollars by wire transfer to a bank in the United States designated in writing by Voyager.

5.13 Taxes.

5.13.1 General. Each Party will be solely responsible for the payment of all taxes imposed on its share of income arising directly or indirectly from the activities of the Parties under this Agreement.

5.13.2 Indirect Tax. All amounts mentioned in this Agreement are exclusive of any value added, goods and services, sales, use, excise, consumption and other similar indirect Taxes ("Indirect Taxes"). Where the prevailing legislation requires the recipient to self-account for Indirect Taxes (for example, but not limited to, the reverse charge mechanism), then Novartis covenants that it will correctly account for Indirect Taxes in respect of the services received. Voyager shall issue all invoices in full compliance with the Indirect Tax laws and regulations applicable at Voyager's place of business. If any Indirect Taxes are due based on local law, Voyager will be allowed to add the amount of Indirect Taxes to the amounts mentioned in this Agreement and invoice Novartis the net amount plus the applicable Indirect Taxes. Both parties agree that Voyager is in general allowed to issue tax exempt invoices in case of cross-border supply of services as agreed in this contract. Each Party will be responsible for reporting its own transactions to the local tax authorities if required for Indirect Tax purposes. There will be no shared, mutual or otherwise collective Indirect Tax filings that may suggest that the Parties are anything other than separately operational entities for Indirect Tax purposes.

5.13.3 Tax Action. Notwithstanding anything in this Agreement to the contrary, if an action (including any assignment or sublicense of its rights or obligations under this Agreement, relocation of a Party to a different jurisdiction, or any failure to comply with applicable Laws or filing or record retention requirements) by a Party (a "Tax Action") leads to the imposition of withholding tax liability or Indirect Tax on the other Party that would not have been imposed in the absence of such Tax Action or in an increase in such liability above the liability that would have been imposed in the absence of such Tax Action, then the sum payable by that Party (in respect of which such deduction or withholding is required to be made) shall be increased to the extent necessary to ensure that the other Party receives a sum equal to the sum which it would have received had no such Tax Action occurred.

5.13.4 Subject to Section 5.13.3, in the event any payments to be made to Licensor or its Affiliates under this Agreement are subject to withholding tax under applicable Laws, including extra-territorial taxation, or if it is unclear whether the requirements of applicable Laws, including extra-territorial taxation, are met, Novartis or its Affiliates shall be authorized to deduct the withholding tax from the payments, and shall pay all such withholding tax to the relevant tax authority, so that only the correspondingly reduced amount of payments (i.e. the full amount

payable less withholding tax) is paid out to Licensor. Novartis shall provide Licensor with proof of the withholding tax payment. Any such withholding taxes required under applicable Law to be paid or withheld shall be an expense of, and borne solely by, Voyager. If a Party believes that it is required to withhold taxes on a payment to the other Party, the paying Party shall use Commercially Reasonable Efforts to notify the other Party of such determination no less than [**] prior to making such payment (but notice shall not be required for subsequent payments except in case of changes to the expected withholding). Novartis will provide Voyager with reasonable assistance to enable Voyager to recover such taxes as permitted by Law.

5.13.5 Licensor and Novartis shall make all reasonable efforts to obtain relief or reduction of withholding tax under the applicable tax treaties, including but not limited to the submission or issuance of requisite forms and information. If a special procedure is required for treaty relief by Law, a treaty relief based on a tax treaty will only be taken into account if Licensor submits any exemption certificate requested by Novartis to Novartis in accordance with legal requirements on or prior to the time of the payment to Licensor.

5.13.6 If no withholding tax deduction has been made on the payments to Voyager or its Affiliates under this Agreement, but tax authorities subsequently take the position that a withholding tax deduction should have been made, including extra-territorial taxation, Voyager shall provide, at its own expense, all reasonable support to Novartis to obtain any available relief or reduction of withholding under the applicable Laws, including but not limited to the submission or issuance of requisite forms and information, and the Parties will bear such liability (reimburse one another as necessary) in a manner consistent with that which would have resulted had the tax been originally withheld. Any refunds of withholding taxes that are granted to Voyager by the competent tax authority and which would cause Voyager to receive payments in excess of that which Novartis would owe under this Agreement, including related interest, shall be paid to Novartis by Voyager.

5.13.7 Late Payments. Any amount required to be paid by a Party hereunder which is not paid on the date due shall bear interest compounded daily, to the extent permitted by law, at the Federal Funds Effective Rate EFFR or any successor to such rate) for the date such payment was due, as reported by the Federal Reserve of New York (https://apps.newyorkfed.org/markets/autorates/fed%20funds).

ARTICLE 6
INTELLECTUAL PROPERTY RIGHTS

6.1 Ownership; Disclosure.

6.1.1 Novartis Background IP. As between the Parties, Novartis will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Novartis and existing as of or before the Effective Date; or (b) Invented, developed, created, generated or acquired solely by or on behalf of Novartis after the Effective Date((a) and (b), collectively, "Novartis Background IP").

6.1.2 Voyager Background IP. As between the Parties, Voyager will own and Control all right, title and interest in and to all Patents or Know-How: (a) Controlled by Voyager and existing as of or before the Effective Date; or (b) Invented, developed, created, generated or acquired solely by or on behalf of Voyager after the Effective Date ((a) and (b), collectively, "Voyager Background IP").

6.1.3 Joint Inventions. Subject to Section 6.2.3, ownership of any Patents and Know-How that are Invented or otherwise developed jointly by or on behalf of the Parties during Term and in the course of the Parties' activities under this Agreement ("Joint Inventions") will follow inventorship under U.S. patent law.

6.2 Patent Prosecution and Maintenance; Defense Proceedings.

6.2.1 Capsid Patents; Licensed Capsid Patents.

(a) Prior to Novartis's exercise of an Option, Voyager will have the sole obligation, at its sole cost and expense (except as otherwise provided herein), to Prosecute and Maintain the Capsid Patents and for conducting any Defense Proceeding with respect to the Capsid Patents, and will have sole decision-making authority with respect to matters relating to the Prosecution and Maintenance or the conduct of Defense Proceedings for the Capsid Patents. Voyager will: (i) allow Novartis a reasonable opportunity and reasonable time to review and provide comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager in relation to Capsid Patents that Cover Capsid Candidates under Evaluation by Novartis before any applicable filings are submitted to any relevant patent office and (ii) reasonably consider any reasonable and timely comments offered by Novartis in any final filings submitted by Voyager to any relevant patent office in relation to such Capsid Patents; provided that Novartis will not have any right to review or comment on any Capsid Patent application prior to filing of such application with the relevant patent office. Voyager will not disclose in, or in connection with Prosecution of, any Capsid Patent any of Novartis's Confidential Information without the prior written consent of Novartis.

6.2.2 Licensed Capsid Patents. Following Novartis's exercise of an Option:

(a) Voyager will have the sole right (but not the obligation), at its sole cost and expense (except as otherwise provided herein), (i) to Prosecute and Maintain the Licensed Capsid Patents and (ii) for conducting any Defense Proceeding with respect to the Licensed Capsid Patents, subject to Novartis's comment rights set forth below. Upon Novartis's request, Voyager will reasonably consider filing, Prosecuting and Maintaining the Licensed Capsid Patents in any jurisdiction reasonably requested by Novartis including consideration of an arrangement in which Novartis pays Voyager for all of its costs, or a pro-rata share of costs as applicable, for such activity if Voyager would not, but for the Novartis request, otherwise ordinarily perform the activity in such jurisdiction. Following Novartis's exercise of an Option, The Parties will coordinate to develop a patent strategy designed to maximize the value and coverage of the Licensed Capsid Patents for the associated Licensed Products. Novartis will have sole authority to make decisions for a patent term extension (e.g., selection of which patents to apply for patent term extension) in

respect to any Licensed Products pursuant to rights under the Drug Price Competition and Patent Term Restoration Act, 21 U.S.C. §355, as amended (or any successor statute or regulation) in the U.S., and pursuant to any analogous Law in a foreign jurisdiction; provided, however, that Novartis may not elect to file for such patent term extension on a Licensed Capsid Patent Controlled by Voyager without Voyager's prior written consent, not to be unreasonably withheld, conditioned or delayed. For the avoidance of doubt, Patents assigned to Voyager pursuant to Section 6.2.3(b) are not considered Controlled by Voyager for this purpose.

(b) Voyager will have sole decision-making authority with respect to matters relating to the Prosecution and Maintenance or the conduct of Defense Proceedings for the Capsid Patent(s) or the Licensed Capsid Patent(s), including any decisions to terminally disclaim a Patent in which Voyager has an interest.

(c) With regard to the Licensed Capsid Patents, Voyager will: (i) allow Novartis a reasonable opportunity and reasonable time to review and provide comment to Voyager's in-house counsel regarding relevant substantive communications by Voyager and drafts of any responses or other proposed substantive filings by Voyager before any applicable filings are submitted to any relevant patent office and (ii) give due consideration to any reasonable and timely comments offered by Novartis in any final filings, including terminal disclaimers, submitted by Voyager to any relevant patent office. Voyager will not disclose in, or in connection with prosecution of, any Licensed Capsid Patent any of Novartis's Confidential Information without the prior written consent of Novartis.

6.2.3 Licensed Product Patents.

(a) As between the Parties, and subject to Section 6.2.2(c), Novartis will own and Control all right, title, and interest in and to all Licensed Product Patents.

(b) Novartis shall not file a Licensed Product Patent prior to the first publication of any Capsid Patent that first discloses the sequence for any Capsid or Licensed Capsid that is the subject of the corresponding Licensed Product, without first receiving Voyager's written approval, not to be unreasonably withheld, conditioned or delayed, to make such filing. In addition to other provisions that the Parties may agree are appropriate to implement, in the event that: (i) a Licensed Product Patent is filed after Voyager's approval in accordance with (b)) or (ii) any other Licensed Product Patent filed by Novartis creates an obviousness-type double patenting (OTDP) rejection or challenge against a Capsid Patent and that requires filing of a terminal disclaimer to obviate such rejection or challenge (and cannot otherwise be overcome by other approaches as agreed to by the Parties), Novartis will assign its right, title, and interest in such Licensed Product Patent to Voyager in the United States only, subject to Novartis receiving the exclusive license set forth in Section 3.1.2(b); provided that Novartis will retain the sole right, at its sole cost and expense, (i) to Prosecute and Maintain the Licensed Product Patents in all countries and (ii) for enforcing or defending all assigned Licensed Product Patents.

6.2.4 Joint Patents.

(a) Neither Party will file any Patent application for a Joint Invention without mutual consent. If the Parties decide to seek patent protection for any Joint Invention, the Parties will cooperate in good faith to determine, on a case-by-case basis, which Party will have the responsibility for Prosecuting and Maintaining, and conducting Defense Proceedings relating to any Joint Patents, and how the cost for such activities will be shared.

6.2.5 Cooperation. Each Party will reasonably cooperate with and assist the other Party in connection with the activities of such Party under this Section 6.2 upon the reasonable request of the other Party, including by making scientists and scientific records reasonably available and the execution of all such documents and instruments and the performance of such acts as may be reasonably necessary in order to continue any Prosecution and Maintenance or conduct any Defense Proceedings of such Patents.

6.3 Enforcement.

6.3.1 Notice. Each Party will promptly notify the other Party in writing of any knowledge it acquires of any actual or potential Competitive Infringement by a Third Party (the "Infringement Notice").

6.3.2 Capsid Patents. Unless and until each applicable Option Exercise Date, as between Novartis and Voyager, Voyager will have the sole right, but not the obligation, to institute litigation or take other steps to remedy Competitive Infringement in connection with the Capsid Patents in the Territory, and any such litigation or steps will be at Voyager's expense and all recoveries will be retained by Voyager.

6.3.3 Licensed Capsid Patents. Upon the applicable Option Exercise Date, as between Novartis and Voyager, Voyager will have the first right (but not the obligation), using Commercially Reasonable Efforts, to institute litigation or take other steps to remedy such Competitive Infringement in connection with the Licensed Capsid Patents in the Territory, and any such litigation or steps will be at Voyager's expense and all recoveries will be retained by Voyager. In the event that (a)(i) Voyager (A) does not institute litigation or take other steps to remedy such Competitive Infringement in connection with the Licensed Capsid Patent within [**] after the corresponding Competitive Infringement is first identified, or (B) does not continue its litigation to a final, unappealable decision, or (B) does not remedy the Competitive Infringement through other means within such [**] period, and (ii) such Competitive Infringement has (or reasonably threatens to have) a direct and material adverse impact on Novartis's Commercialization of Licensed Products, then (b) the royalties due to Voyager pursuant to Section 5.6 and payable as of the date of the Infringement Notice shall be reduced by [**] percent ([**]%), but only in the country in which the infringing activity exists with no right of offset with regard to royalties payable for other jurisdictions.

6.3.4 Licensed Product Patents. As between Novartis and Voyager, Novartis will have the sole right, but no obligation, to take action to obtain a discontinuance of infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any Licensed Product Patent in the Territory, and any such litigation or steps will be at Novartis's expense and all recoveries will be retained by Novartis.

6.3.5 Joint Patents. Immediately after an infringement of a Joint Patent is first identified, the Parties shall meet and cooperate in good faith to determine, on a case-by-case basis, (i) what action, if any, the Parties will take to obtain a discontinuance of such infringement or bring suit against a Third Party infringing or challenging the validity or enforceability of any Joint Patent, and (ii) how the costs for and any recoveries from such activities will be shared.

6.4 Infringement Claimed by Third Parties.

6.4.1 Notice. If a Third Party commences, or threatens to commence, any proceeding against a Party alleging infringement of such Third Party's intellectual property by the Exploitation by a Party, its Affiliates, subcontractors or Sublicensees of any Licensed Product, the Party against whom such proceeding is threatened or commenced will give prompt notice to the other Party.

6.4.2 Control of Proceeding. Unless the Party against whom such proceeding is filed seeks indemnification for such claim under Article 9, such Party will control the defense and settlement of any such proceeding described in Section 6.4.1 at its own cost and expense, using counsel of its choice, in its sole discretion. If the Party against whom such proceeding is filed does seek indemnification for such claim, then the provisions of Article 9 will govern the Parties' rights and responsibilities with respect to such claim.

ARTICLE 7
CONFIDENTIALITY

7.1 Confidentiality; Exceptions. Except to the extent expressly authorized by this Agreement, the Parties agree that the receiving Party (the "Receiving Party") will keep confidential and will not publish or otherwise disclose or use for any purpose other than to perform its obligations and exercise its rights as provided for in this Agreement any Know-How or other confidential and proprietary information and materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise) that is disclosed to it by the other Party (the "Disclosing Party"), including trade secrets, Know-How, inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the Disclosing Party's past, present or future marketing, financial, or Exploitation activities of any product or potential product or technology of the Disclosing Party or the pricing thereof (collectively, "Confidential Information"). For clarity, any data, information, or Patent filings provided by one Party to the other Party will constitute the Disclosing Party's Confidential Information. Without limiting the foregoing, the Receiving Party will treat all Confidential Information provided by the Disclosing Party with the same degree of care as the Receiving Party uses for its own similar information, but in no event less than a reasonable degree of care. Notwithstanding the foregoing, "Confidential Information" will exclude information to the extent that it can be established by the Receiving Party that such information:

7.1.1 was in the lawful knowledge or possession of the Receiving Party prior to the time it was first disclosed to the Receiving Party by the Disclosing Party, or was otherwise developed independently by the Receiving Party without reference to any of the Disclosing Party's

Confidential Information, as evidenced by written records kept in the ordinary course of business, or other documentary proof of knowledge or possession by the Receiving Party;

7.1.2 was generally available to the public or otherwise part of the public domain at the time of its first disclosure to the Receiving Party by the Disclosing Party;

7.1.3 became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party by the Disclosing Party and other than through any act or omission of the Receiving Party in breach of this Agreement or the Existing Confidentiality Agreement; or

7.1.4 was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

Any information disclosed by a Party to the other Party prior to the Effective Date pursuant to the Mutual Confidential Disclosure Agreement between Parties dated [**] (the "Existing Confidentiality Agreement"), that was considered Confidential Information (as defined in the Existing Confidentiality Agreement) will be Confidential Information of such Disclosing Party hereunder, subject to the provisions of Sections 7.1.1, 7.1.2, 7.1.3, and 7.1.4. The existence and terms of this Agreement will be considered the Confidential Information of both Parties. Any reports, Know-How, and other proprietary or sensitive information disclosed or shared by one Party with the other Party pursuant to the activities contemplated by this Agreement will be the Confidential Information of the Party that first shared such report, Know-How or other proprietary or sensitive information with the other Party.

7.2 Authorized Disclosure.

7.2.1 Disclosure to a Party's Representatives. Notwithstanding the foregoing provisions of Section 7.1, the Receiving Party may disclose Confidential Information belonging to the Disclosing Party to the Receiving Party's Representatives who (a) have a need to know such Confidential Information in connection with the performance of the Receiving Party's obligations or the exercise of the Receiving Party's rights under this Agreement and (b) have agreed in writing to non-disclosure and non-use provisions with respect to such Confidential Information that are at least as restrictive as those set forth in this Article 7.

7.2.2 Disclosure to Third Parties. Notwithstanding Section 7.1, each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary:

(a) to Governmental Authorities (i) to the extent desirable to obtain or maintain INDs or Regulatory Approvals for any Licensed Product within the Territory and (ii) in order to respond to inquiries, requests or investigations relating to Licensed Products or this Agreement;

(b) to existing or prospective outside consultants, contractors, advisory boards, investors, collaboration partners, professional advisors, managed care organizations, and

non-clinical and clinical investigators, in each case to the extent desirable to develop, register or market any Licensed Product or otherwise as reasonably necessary to perform such Party's obligations under this Agreement; provided that the Receiving Party shall obtain the same confidentiality obligations from such Third Parties as it obtains with respect to its own similar types of confidential information;

(c) in connection with filing or prosecuting Patent rights or trademark rights as permitted by this Agreement;

(d) in connection with prosecuting or defending litigation as permitted by this Agreement;

(e) subject to the provisions of Section 7.5, in connection with or included in scientific presentations and publications relating to Compounds or Products, including abstracts, posters, journal articles and the like, and posting results of and other information about clinical trials to clincialtrials.gov or PhRMA websites;

(f) to a court or arbitrator, to the extent reasonably necessary in order to enforce its rights under this Agreement;

(g) in communication with existing or prospective investors, lenders, professional advisors, acquirers, merger partners, collaboration partners, subcontractors, Sublicensees, or licensees on a need to know basis, in each case under appropriate confidentiality obligations substantially equivalent to those of this Agreement; or

(h) to the extent mutually agreed to in writing by the Parties.

7.3 Residual Knowledge Exception. Notwithstanding any provision of this Agreement to the contrary, Residual Knowledge shall not be considered Confidential Information for purposes of this Article 7.

7.4 Press Release; Disclosure of Agreement.

7.4.1 Press Releases. On or promptly after the Effective Date, the Parties anticipate issuing a public announcement regarding the signing of this Agreement in a form to be agreed by the Parties. Except as may be expressly permitted under Section 7.4.2, neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party; provided that to the extent information regarding this Agreement has already been publicly disclosed, except as a result of a breach of this Agreement, each Party may subsequently disclose the same information to the public without the consent of the other Party, provided that such information remains true, accurate, and up to date. In addition, nothing in this Agreement shall prevent Novartis from making any scientific publication or public announcement with respect to any Licensed Product under this Agreement; *provided, however*, that, except as permitted under Section 7.2.1, Novartis shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so.

7.4.2 SEC Filings and other Disclosures of this Agreement. Notwithstanding Section 7.4.1, each Party will be permitted to disclose the existence and terms of this Agreement to the extent required to comply with applicable Laws including the rules or regulations of the U.S. Securities and Exchange Commission, or similar agency in any country other than the United States, or of any stock exchange, including Nasdaq, provided that (a) prior to disclosing this Agreement or any of the terms hereof as permitted under this Section 7.4.2, the Parties will coordinate in advance with each other in connection with the redaction of certain provisions of this Agreement prior to such disclosure (the "Redacted Version"), (b) to the extent permitted by applicable Laws, the Parties will use reasonable efforts to file redacted versions with such agencies and stock exchanges that are consistent with the Redacted Version, and (c) each Party will, at its own expense, use reasonable efforts to seek confidential treatment for such terms as may be reasonably requested by the other Party.

7.5 Publications. On a Capsid Candidate-by-Capsid Candidate basis, prior to exercising its Option for a particular Capsid Candidate, Novartis will not publish or publicly disclose the scientific results of any of the Evaluation conducted by it for such Capsid Candidate, without the prior written consent of Voyager. Following the Option Exercise Date for a particular Licensed Capsid, nothing in this Agreement shall prevent Novartis from making any scientific publication or public announcement with respect to any Licensed Product containing such Licensed Capsid; provided, however, that, except as permitted under Section 7.2, Novartis shall not disclose any of Voyager's Confidential Information in any such publication or announcement without obtaining Voyager's prior written consent to do so. In addition, (i) Voyager shall not publish or make any public announcement regarding a Licensed Product without Novartis's prior written approval, and (ii) Novartis shall provide Voyager a copy of each publication or other public disclosure relating to a Licensed Product that contains unpublished information relating to a Licensed Capsid. During the Term, each Party will provide the other Party (the "Non-Disclosing Party") for review and approval any proposed abstract, manuscript, or presentation that contains the Non-Disclosing Party's Confidential Information. Written copies of each proposed publication that are required to be submitted hereunder shall be provided to the Non-Disclosing Party no less than [**] with respect to disclosures in a patent application) prior to its intended submission for publication or presentation. The Non-Disclosing Party will respond in writing promptly and in no event later than [**] after receipt of the proposed publication or presentation, with one or more of the following: (a) comments on the proposed publication or presentation, which the publishing Party will consider in good faith and use reasonable efforts to incorporate, (b) a specific statement of concern, based upon the need to delay publication if the Non-Disclosing Party determines that the proposed publication or presentation contains or describes intellectual property that needs to be incorporated into a Patent application; provided that such delay shall not exceed an additional [**] unless agreed in writing by the Parties, or (c) an identification of the Non-Disclosing Party's Confidential Information that needs to be removed from the proposed publication or presentation.

7.6 Remedies. Each Party will be entitled to seek, in addition to any other right or remedy it may have, at Law or in equity, a temporary injunction, without the posting of any bond or other security, enjoining or restraining the other Party from any violation or threatened violation of this Article 7.

ARTICLE 8
REPRESENTATIONS AND WARRANTIES

8.1 Representations and Warranties of Both Parties. Each Party hereby represents and warrants to the other Party, as of the Effective Date, that:

8.1.1 such Party is duly organized, validly existing and in good standing under the Laws of the jurisdiction of its incorporation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

8.1.2 such Party has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder and does not require any action or approval by any of its shareholders or other holders of its voting securities or voting interests;

8.1.3 this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against it in accordance with the terms hereof;

8.1.4 the execution, delivery and performance of this Agreement by such Party does not conflict with any agreement or any provision thereof, or any instrument or understanding, oral or written, to which it is a party or by which it is bound, nor violate any law or regulation of any court, governmental body or administrative or other agency having jurisdiction over such Party;

8.1.5 neither such Party nor any of its Affiliates has been debarred or is subject to debarment pursuant to Section 306 of the FD&C Act, as amended, or that is the subject of a conviction described in such section; and

8.1.6 no government authorization, consent, approval, license, exemption of or filing or registration with any court or governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, under any applicable Laws currently in effect, is or will be necessary for, or in connection with, the transaction contemplated by this Agreement or any other agreement or instrument executed in connection herewith, or for the performance by it of its obligations under this Agreement.

8.2 Representations and Warranties, as applicable, of Voyager. Voyager hereby represents, warrants, and covenants to Novartis, as of the Effective Date that:

8.2.1 Voyager has disclosed to Novartis all material scientific and technical information and all material information that, to Voyager's Knowledge, are relevant to safety and efficacy with respect to the Capsids;

8.2.2 (a) Schedule 1.22 sets forth a true and complete list of all Capsid Patents as of the Effective Date that Cover the Capsid Candidates (the "Relevant Capsid Patents"), (b) each such Patent remains in full force and effect and (c) Voyager or its Affiliates have timely paid all filing and renewal fees payable with respect to such Patents;

8.2.3 Voyager is the sole and exclusive owner of the Relevant Capsid Patents and Voyager's Know-How, all of which is free and clear of any claims, liens, charges, or encumbrances that would conflict with the rights granted to Novartis hereunder;

8.2.4 Voyager has and will have the right, power, and authority to grant all rights, title, and interests in the licenses granted or to be granted to Novartis under this Agreement;

8.2.5 Voyager has not granted any right or license, to any Third Party relating to any of the Relevant Capsid Patents that would conflict with the rights or licenses granted to Novartis hereunder as of the Effective Date;

8.2.6 [**], no claim, demand, suit, proceeding, arbitration, inquiry, investigation, litigation, or other legal action of any nature, civil, criminal, regulatory or otherwise, is pending, has been brought, or to Voyager's Knowledge, threatened against Voyager or any Affiliate of Voyager, or, to Voyager's Knowledge, any Third Party, alleging that the Exploitation of Voyager's Background IP is infringing or, if practiced or commercialized, will infringe the rights of any Third Party;

8.2.7 there is no judgment or settlement against or owed by Voyager or any of its Affiliates, in each case in connection with the Relevant Capsid Patents or Voyager Know-How relating to the transactions contemplated by this Agreement;

8.2.8 to Voyager's Knowledge, no Third-Party has challenged or threatened to challenge the scope, validity or enforceability of any Relevant Capsid Patents (including, by way of example, through the institution or written threat of institution of interference, nullity or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous foreign Governmental Authority;

8.2.9 to Voyager's Knowledge: (a) the Relevant Capsid Patents, are, or, upon issuance, will be, valid and enforceable patents; and (b) as of the Effective Date no Person is infringing or threatening to infringe, or misappropriating or threatening to misappropriate, the Relevant Capsid Patents in a manner that would affect Novartis's rights under this Agreement;

8.2.10 all of its employees, officers, and consultants have executed (a) valid and enforceable agreements assigning or (b) have existing obligations under applicable Laws requiring assignment to Voyager of all Inventions made during the course of and as the result of their association with Voyager and obligating the individual to maintain as confidential Voyager's Confidential Information as well as confidential information of other Persons (including Novartis and its Affiliates) which such individual may receive;

8.2.11 Voyager has taken reasonable precautions to preserve the confidentiality of any Know-How that constitutes Voyager's Background IP existing as of the Effective Date and that would be licensed to Novartis upon exercise of any Option, including requiring each Person having access to any Know-How within such Voyager's Background IP to be subject to confidentiality, non-use and non-disclosure obligations protecting such Know-How as the confidential, proprietary materials and information of Voyager;

8.2.12 to Voyager's Knowledge, Voyager has complied with all applicable Laws, including any disclosure requirements, in connection with the filing, prosecution, and maintenance of the Relevant Capsid Patents;

8.2.13 to Voyager's Knowledge, Voyager has independently developed all Voyager Know-How or otherwise has a valid right to use, and to permit Novartis, Novartis' Affiliates, and Novartis' Sublicensees to use, the Voyager Know-How for all permitted purposes under this Agreement;

8.2.14 no Relevant Capsid Patent is subject to any funding agreement with any government or Governmental Authority;

8.2.15 neither Voyager nor any of its Affiliates are party to or otherwise subject to any agreement or arrangement that would conflict with Novartis's rights or Voyager's obligations under this Agreement;

8.2.16 [**], to the best of Voyager's Knowledge, the Exploitation by Voyager or Novartis (or their respective Affiliates or Sublicensees) of any Capsid Candidate does not infringe any claim of an issued patent of any Third Party as of the Effective Date;

8.2.17 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied in all material respects with all applicable Law and accepted pharmaceutical industry business practices with regard to the subject matter of this Agreement, including, to the extent applicable, the FD&C Act (21 U.S.C. § 301, et seq.), the Anti-Kickback Statute (42 U.S.C. § 1320a-7b), Civil Monetary Penalty Statute (42 U.S.C. § 1320a-7a), the False Claims Act (31 U.S.C. § 3729 et seq.), comparable state statutes, the regulations promulgated under all such statutes, and the regulations issued by the FDA, consistent with the 'Compliance Program Guidance for Pharmaceutical Manufacturers' published by the Office of Inspector General, U.S. Department of Health and Human Services;

8.2.18 with respect to any Licensed Capsids, payments, or services provided under this Agreement, Voyager, its Affiliates, and to its Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have not taken and will not during the Term take any action directly or indirectly to offer, promise or pay, or authorize the offer or payment of, any money or anything of value in order to improperly or corruptly seek to influence any government official or any other person in order to gain an improper advantage, and has not accepted, and will not accept in the future such payment; and

8.2.19 Voyager, its Affiliates, and to Voyager's Knowledge all Third Parties and Representatives acting on Voyager's behalf, have complied with the laws and regulations of the countries where it operates, including anti-bribery and anti-corruption laws, including, to the extent applicable, the U.S. Foreign Corrupt Practices Act of 1977 and the U.K. Bribery Act 2010, accounting and record keeping laws, and laws relating to interactions with healthcare professionals or healthcare providers and government officials.

8.3 Mutual Covenants. Each Party hereby covenants to the other Party that, from the Effective Date until expiration or termination of this Agreement:

8.3.1 it will perform its obligations under this Agreement in compliance with applicable Laws;

8.3.2 All individuals who are employees or independent contractors of such Party or any of its Affiliates working under this Agreement will be under the obligation to assign or exclusively license all right, title and interest in and to their Know-How, and all intellectual property rights therein, to such Party or its Affiliate as the sole owner or exclusive licensee thereof;

8.3.3 such Party will not knowingly (a) employ, or use any contractor or consultant that employs or uses, any Person debarred or disqualified by the FDA (or subject to a similar sanction of EMA or any other Governmental Authority) or, (b) employ any Person that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA or any other Governmental Authority), in each of clauses (a) and (b) in the conduct of its activities under this Agreement; and

8.3.4 in performing its obligations or exercising its rights under this Agreement, such Party, its Affiliates, and, with respect to Novartis, its Sublicensees, will comply with all applicable Law, including all anti-corruption Laws.

8.4 Voyager Covenants. In addition to the covenants made by Voyager elsewhere in this Agreement, Voyager hereby covenants to Novartis that:

8.4.1 during the Research Term: (a) other than the conduct of the Campaigns, Voyager will not conduct any internal program or program on behalf of a Third Party that is directed to Development or Commercialization of any Capsid Candidates for use in the Licensed Field any therapeutic product comprising a Capsid Candidate or Licensed Capsid in combination with a payload intended to have a therapeutic effect on a Subject Target when packaged into a Capsid and delivered to the appropriate cells; and (b) Voyager will not grant any Third Party or Affiliate any right or license (including options) under Voyager's rights in any Capsid Candidate or Licensed Capsid to Exploit in the Licensed Field any therapeutic product comprising a Capsid Candidate or Licensed Capsid in combination with a payload intended to have a therapeutic effect on a Subject Target when packaged into a Capsid and delivered to the appropriate cells.

8.4.2 from and after the applicable Option Exercise Date, during the Term, Voyager shall not, and shall cause its Affiliates not to: (a) license, sell, assign or otherwise transfer to any Person (other than Novartis or its Affiliates or Sublicensees pursuant to the terms of this Agreement) any rights under the Licensed Capsid Patents to use a Licensed Capsid with the corresponding a Subject Target (or agree to do any of the foregoing) or (b) incur or permit to exist, with respect to any Licensed Capsid Patents, any lien, encumbrance, charge, security interest, mortgage, liability, assignment, grant of license or other binding obligation that is or would be inconsistent with the licenses and other rights granted to Novartis or its Affiliates under this Agreement;

8.4.3 during the Term, Voyager will: (a) not enter into any agreement with a Third-Party that conflicts with (i) the rights granted to Novartis under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; (b) not amend or otherwise modify any agreements with a Third Party or consent or waive rights with respect thereto in any manner that conflicts with (i) the rights granted to Novartis under this Agreement or (ii) Voyager's ability to fully perform its obligations hereunder; and

8.4.4 during the Term, Voyager will maintain valid and enforceable agreements with all Persons acting by or on behalf of Voyager or its Affiliates under this Agreement which require such Persons to assign to Voyager their entire right, title and interest in and to all Licensed Capsid Patents and Voyager's Know-How.

8.5 Representation by Legal Counsel. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

8.6 Disclaimer. Except as otherwise expressly set forth in this Agreement, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, ALL OF WHICH ARE HEREBY SPECIFICALLY EXCLUDED AND DISCLAIMED.

ARTICLE 9
INDEMNIFICATION; INSURANCE

9.1 Indemnification by Novartis. Novartis will indemnify, hold harmless and defend Voyager and its Affiliates, and its or their respective directors, officers, employees, agents, consultants and Representatives (each a "Voyager Indemnified Party"), from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys (collectively, "Losses") that the Voyager Indemnified Party may be required to pay to one or more Third Parties to the extent arising out of or resulting from any Third Party suits, claims, actions, proceedings, hearings, investigations, judgments, orders, decrees, stipulations, or injunctions or demands ("Third Party Claims") arising out of or resulting from:

9.1.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Novartis or any of its Affiliates or Sublicensees, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Novartis or exercise of Novartis's rights under this Agreement;

9.1.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Novartis; or

9.1.3 the Exploitation of any Licensed Product conducted by or on behalf of Novartis, any of its Affiliates or any Sublicensee hereunder, including: (a) any product liability,

personal injury, property damage or other damage; and (b) infringement of any Patent or other intellectual property right of any Third Party; except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Voyager Indemnified Party, or (y) any Third Party Claim for which Voyager is responsible for indemnifying Novartis pursuant to Section 9.2.

9.2 Indemnification by Voyager. Voyager will indemnify, hold harmless and defend, Novartis and its Affiliates, and its or their respective directors, officers, employees, consultants, agents, and Representatives (each a "Novartis Indemnified Party"), from and against any and all Losses that the Novartis Indemnified Party may be required to pay to one or more Third Parties, to the extent arising out of or resulting from any Third Party Claims arising out of or resulting from:

9.2.1 the gross negligence, recklessness or wrongful intentional acts or omissions of Voyager or any of its Affiliates or subcontractors, or its or their respective directors, officers, employees, agents, consultants or Representatives, in connection with performance by or on behalf of Voyager or exercise of Voyager's rights under this Agreement; or

9.2.2 any material breach of this Agreement, including any representation, warranty, or covenant, by Voyager; except, in each case, to the extent such Losses arise from (x) the negligence, recklessness, or intentional acts of any Novartis Indemnified Party or (y) any Third Party Claim for which Novartis is responsible for indemnifying Voyager pursuant to Section 9.1.

9.3 Notice. Each Party will notify the other Party in writing in the event it becomes aware of a claim for which indemnification may be sought hereunder. In the event that any Third Party asserts a Claim or other proceeding (including any governmental investigation) with respect to any Third Party Claim for which a Party (the "Indemnified Party") is entitled to indemnification hereunder, then the Indemnified Party shall promptly notify the Party obligated to indemnify the Indemnified Party (the "Indemnifying Party") thereof; *provided, however,* that no delay on the part of the Indemnified Party in notifying the Indemnifying Party shall relieve the Indemnifying Party from any obligation hereunder unless (and then only to the extent that) the Indemnifying Party is prejudiced thereby.

9.4 Control. Subject to each Party's right to control certain actions described in Sections 6.3 and 6.4 (even where such Party is the Indemnifying Party), the Indemnifying Party shall have the right, exercisable by notice to the Indemnified Party within [**] after receipt of notice from the Indemnified Party of the commencement of or assertion of any Third Party Claim, to assume direction and control of the defense, litigation, settlement, appeal, or other disposition of the Third Party Claim (including the right to settle the claim solely for monetary consideration) with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party; provided that (a) the Indemnifying Party has sufficient financial resources, in the reasonable judgment of the Indemnified Party, to satisfy the amount of any adverse monetary judgment that is sought, (b) the Third Party Claim seeks solely monetary damages, and (c) the Indemnifying Party expressly agrees in writing that as between the Indemnifying Party and the Indemnified Party, the Indemnifying Party shall be solely obligated to satisfy and discharge the Third Party Claim in full (the conditions set forth in clauses (a), (b), and (c) above are collectively referred to

as the "Litigation Conditions"). Within [**] after the Indemnifying Party has given notice to the Indemnified Party of its exercise of its right to defend a Third Party Claim, the Indemnified Party shall give notice to the Indemnifying Party of any objection thereto based upon the Litigation Conditions. If the Indemnified Party reasonably so objects, the Indemnified Party shall continue to defend the Third Party Claim, at the expense of the Indemnifying Party, until such time as such objection is withdrawn. If no such notice is given, or if any such objection is withdrawn, the Indemnifying Party shall be entitled, at its sole cost and expense, to assume direction and control of such defense, with counsel selected by the Indemnifying Party and reasonably acceptable to the Indemnified Party. During such time as the Indemnifying Party is controlling the defense of such Third Party Claim, the Indemnified Party shall cooperate, and shall cause its Affiliates and agents to cooperate upon request of the Indemnifying Party, in the defense or prosecution of the Third Party Claim, including by furnishing such records, information, and testimony and attending such conferences, discovery proceedings, hearings, trials, or appeals as may reasonably be requested by the Indemnifying Party. In the event that the Indemnifying Party does not satisfy the Litigation Conditions or does not notify the Indemnified Party of the Indemnifying Party's intent to defend any Third Party Claim within [**] after notice thereof, the Indemnified Party may (without further notice to the Indemnifying Party) undertake the defense thereof with counsel of its choice and at the Indemnifying Party's expense (including reasonable, out-of-pocket attorneys' fees and costs and expenses of enforcement or defense). The Indemnifying Party or the Indemnified Party, as the case may be, shall have the right to join in (including the right to conduct discovery, interview, and examine witnesses and participate in all settlement conferences), but not control, at its own expense, the defense of any Third Party Claim that the other Party is defending as provided in this Agreement.

9.5 Settlement. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party (not to be unreasonably withheld, conditioned or delayed), enter into any compromise or settlement that commits the Indemnified Party to take, or to forbear to take, any action (other than the payment of money which will be fully satisfied by the Indemnifying Party). The Indemnified Party shall have the sole and exclusive right to settle any Third Party Claim, on such terms and conditions as it deems reasonably appropriate, to the extent such Third Party Claim involves equitable or other non-monetary relief, but shall not have the right to settle such Third Party Claim to the extent such Third Party Claim involves monetary damages for which the Indemnifying Party would be responsible without the prior written consent of the Indemnifying Party (not to be unreasonably withheld). Each of the Indemnifying Party and the Indemnified Party shall not make any admission of liability in respect of any Third Party Claim without the prior written consent of the other Party, and the Indemnified Party shall use reasonable efforts to mitigate Liabilities arising from such Third Party Claim.

9.6 Insurance. Each Party agrees to obtain and maintain, during the Term, commercial general liability insurance, including products liability insurance (or clinical trials insurance, if applicable), with minimum "A-" A.M. Best rated insurance carriers to cover its indemnification obligations under Section 9.1 or Section 9.2, as applicable, in each case with limits of not less than $[**] U.S. dollars) per occurrence and in the aggregate. All deductibles and retentions will be the responsibility of the named insured. Novartis and its Affiliates will be an additional insured on the Voyager's commercial general liability and products liability policies (or clinical trials insurance, if applicable), and be provided with a waiver of subrogation. For U.S. exposures, additional insured

status Voyager's commercial general liability and products liability policies shall be via form CG20101185 or its equivalent. Licensed Products liability coverage shall be maintained for [**] following termination of this Agreement. To the extent of its culpability or negligence, all coverages of Voyager will be primary and non-contributing with any similar insurance, carried by Novartis. Notwithstanding any provision of this Section 9.6 to the contrary, Novartis may meet its obligations under this Section 9.6 through any combination of insurance and self-insurance. Neither Party's insurance will be construed to create a limit of liability with respect to its indemnification obligations under this Article 9. Each Party will furnish the other Party with a certificate of such insurance promptly following request.

9.7 Limitation of Liability. EXCEPT FOR A BREACH OF ARTICLE 6, ARTICLE 7 OR FOR CLAIMS OF A THIRD PARTY THAT ARE SUBJECT TO INDEMNIFICATION UNDER THIS ARTICLE 9, NEITHER VOYAGER NOR NOVARTIS, NOR ANY OF THEIR RESPECTIVE AFFILIATES, LICENSORS, LICENSEES OR SUBLICENSEES, WILL BE LIABLE UNDER THIS AGREEMENT TO THE OTHER PARTY, ITS AFFILIATES OR REPRESENTATIVES FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL OR PUNITIVE DAMAGES OR LOST PROFITS OR ROYALTIES, LOST DATA OR COST OF PROCUREMENT OF SUBSTITUTE GOODS OR SERVICES, WHETHER LIABILITY IS ASSERTED IN CONTRACT, TORT (INCLUDING NEGLIGENCE AND STRICT LIABILITY), INDEMNITY OR CONTRIBUTION, AND IRRESPECTIVE OF WHETHER THAT PARTY OR ANY REPRESENTATIVE OF THAT PARTY HAS BEEN ADVISED OF, OR OTHERWISE MIGHT HAVE ANTICIPATED THE POSSIBILITY OF, ANY SUCH LOSS OR DAMAGE. Without limiting the generality of the foregoing, "consequential damages" will be deemed to include, and neither Party will be liable to the other Party or any of such other Party's Representatives or stockholders for any damages based on or measured by loss of projected or speculative future sales of the Licensed Products, any payment due upon any unachieved Development Milestone Event, any Sales Milestone Payment due upon any unachieved total annual Net Sales level, any unearned royalties or any other unearned, speculative or otherwise contingent payments provided for in this Agreement.

ARTICLE 10
TERM AND TERMINATION

10.1 Term. This Agreement will commence as of the Effective Date and, unless terminated earlier, this Agreement will continue in full force and effect until (a) if no Option is exercised, the first (1st) anniversary of the Effective Date, or (b) if at least one Option is exercised, with respect to any Licensed Product(s) for which the Option is exercised, on a country-by-country basis, the expiration of the last to expire Royalty Term with respect to such Licensed Product in such country in the Territory (the "Term"). Upon expiration of the Royalty Term for any Licensed Product in any country, the licenses granted with respect to the applicable Licensed Product in such country will become fully paid-up and irrevocable.

10.2 Automatic Termination Upon End of Option Exercise Period**.** If Novartis does not exercise an Option by delivering the Option Exercise Notice to Voyager pursuant to Section 2.3.1 and paying the Option Exercise Fee(s) as set forth in Section 5.3, then, effective automatically upon the later of (a) the first day following the Option Exercise Period and the (b) the [**]

following the due date of the Option Exercise Fee, and without further notice on the part of either Party, this Agreement will automatically terminate with regard to the corresponding Capsid Candidate(s).

10.3 Termination for Breach.

10.3.1 Subject to Sections 4.2.6, 4.3, and the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), this Agreement may be terminated by Voyager (a) on a Licensed Product-by-Licensed Product basis, if Novartis is in material breach of its obligations under this Agreement with respect to such Licensed Product, by providing written notice that includes the particulars of the alleged material breach, or (b) in its entirety, if Novartis is in material breach of is obligations under this Agreement with respect to all Licensed Products, by providing written notice that includes the particulars of the alleged material breach, and In either case ((a) or (b)), the material breach remains uncured for [**] (or [**] in the case of nonpayment), measured from the date written notice of such material breach is given to Novartis; provided, however, that if any breach is not reasonably curable within [**] (or [**] in the case of a nonpayment) and if Novartis is making a *bona fide* effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Novartis a reasonable period of time to cure such breach. If the alleged material breach relates to non-payment of any amount due under this Agreement other than the upfront fee payable under Section 5.1 and Option Exercise Fees, the cure period shall be tolled pending resolution of any *bona fide*, good faith dispute between the Parties as to whether such payment is due.

10.3.2 Subject to the dispute resolution provisions of Sections 11.2 and 11.3 (to the extent applicable), Novartis may terminate this Agreement for cause with respect to one or more Licensed Products in one or more countries in the Territory or may terminate this Agreement in its entirety, at any time during the Term, by giving written notice to Voyager in the event that Voyager commits a material breach of its obligations under this Agreement and such material breach remains uncured for [**], measured from the date written notice of such material breach is given to Voyager; provided, however, that if any breach is not reasonably curable within [**] and if Voyager is making a *bona fide* effort to cure such breach, such termination shall be delayed for a time period to be agreed by both Parties in order to permit Voyager a reasonable period of time to cure such breach.

10.3.3 Notwithstanding anything to the contrary in this Agreement and subject to the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), Novartis may terminate this Agreement in whole or relevant part, immediately and without regard to any cure period, if, in Novartis's reasonable opinion, a violation of Global Trade Control Laws has occurred. Any such termination will be deemed for cause under this Section 10.3.3, under which Novartis will not be responsible for any related payments due, even if activities have already occurred. Voyager will be responsible for reimbursing Novartis for any payments due to Novartis under this Agreement that are blocked due to violation of Global Trade Control Laws

10.4 Termination for Compliance with the Law-related Breach. Subject to the dispute resolution provisions of Section 11.2 and 11.3 (to the extent applicable), Novartis may terminate this Agreement if Voyager breaches any of the representations or warranties set forth in Sections

8.2.17 through 8.2.19 or if Novartis learns that improper payments are being or have been made to government officials by Voyager with respect to services performed in connection with this Agreement. Further, in the event of such termination, Voyager shall not be entitled to any further payment, regardless of any activities undertaken or agreements with additional Third Parties entered into prior to termination, and Voyager shall be liable for damages or remedies as provided by law.

10.5 Termination for Convenience. Novartis may terminate this Agreement in its entirety or on a Licensed Product-by-Licensed Product and country-by-country basis for any or no reason upon ninety (90) days' written notice to Voyager.

10.6 Provisions for Insolvency.

10.6.1 Termination Right. Voyager will be deemed a "Debtor" under this Agreement if, at any time during the Term (a) a case is commenced by or against Voyager under the Bankruptcy Code, (b) Voyager files for or is subject to the institution of bankruptcy, reorganization, liquidation or receivership proceedings (other than a case under the Bankruptcy Code), (c) Voyager assigns all or a substantial portion of its assets for the benefit of creditors, (d) a receiver or custodian is appointed for Voyager's business or (e) a substantial portion of Voyager's business is subject to attachment or similar process; provided, however, that in the case of any involuntary case under the Bankruptcy Code, Voyager will not be deemed a Debtor if the case is dismissed within [**] after the commencement thereof. If Voyager is deemed a Debtor, then Novartis may terminate this Agreement by providing written notice to Voyager. If Novartis terminates this Agreement pursuant to this Section 10.6.1, then, in addition to all other rights that it may have at law, Novartis will have the right to offset, against any payment owing to Voyager hereunder, any damages found or agreed by the Parties to be owed by Voyager to Novartis.

10.6.2 Rights to Intellectual Property. All rights and licenses now or hereafter granted by Voyager to Novartis under or pursuant to any Section of this Agreement, including Article 2 and Article 3 hereof, are rights to "intellectual property" (as defined in the Bankruptcy Code). The Parties acknowledge and agree that the payments provided for under Sections 5.1, 5.2, and 5.3 and all other payments by Novartis to Voyager hereunder, other than royalty payments pursuant to Section 5.6, do not constitute royalties within the meaning of Section 365(n) of the Bankruptcy Code or relate to licenses of intellectual property hereunder. If: (a) (i) a case under the Bankruptcy Code is commenced by or against Voyager, (ii) this Agreement is rejected as provided in the Bankruptcy Code and (iii) Novartis elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code; then (b) Voyager (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) will provide to Novartis all intellectual property licensed hereunder, and agrees to grant and hereby grants to Novartis and its Affiliates a right to access and to obtain possession of and to benefit from and, in the case of any chemical or biological material or other tangible item of which there is a fixed or limited quantity, to obtain a pro rata portion of, any "embodiments" of intellectual property pursuant to Section 365(n) of the Bankruptcy Code (which will be deemed to include the Licensed Know-How), and all other embodiments of the intellectual property licensed hereunder. Voyager will not interfere with the exercise by Novartis or its Affiliates of rights and licenses to intellectual property licensed hereunder and embodiments thereof in accordance with this Agreement and agrees to use

Commercially Reasonable Efforts to assist Novartis and its Affiliates to obtain such intellectual property and embodiments thereof in the possession or control of Third Parties as reasonably necessary or desirable for Novartis or its Affiliates or Sublicensees to exercise such rights and licenses in accordance with this Agreement.

10.6.3 No Limitation of Rights. All rights, powers and remedies of Novartis provided in this Section 10.6 are in addition to and not in substitution for any and all other rights, powers and remedies now or hereafter existing at Law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code involving Voyager.

10.7 Effects of Termination.

10.7.1 Effect of Termination

(a) Automatic Termination Upon End of All Research Terms or Termination Prior to Option Exercise Date. On a Capsid Candidate-by-Capsid Candidate basis, in the event this Agreement is terminated pursuant to Section 10.2 or pursuant to any other provision of this Agreement prior to the Option Exercise Date, the following will apply upon the effective date of termination:

(i) Except as otherwise expressly provided herein, all rights and obligations of each Party hereunder will cease with regard to the corresponding Capsid Candidate or in its entirety if the Agreement is terminated for all Capsid Candidates, including, for the avoidance of doubt, all rights under the Option, all rights to conduct the Evaluation, any and all rights and licenses and sublicenses granted by either Party to the other Party hereunder.

(ii) The Parties shall discuss and determine, based on mutual consent, whether to seek or continue to seek Patent protection with respect to any data generated from the Evaluation and, if applicable, each Party's rights and obligations with respect to such activities. If the Parties cannot reach mutual written agreement on the course of action to take with respect to the filing, prosecution or maintenance such Patent, neither Party will have any responsibility to file, prosecute or maintain such Patent or share in the costs thereof.

(b) Termination for Cause by Voyager; Termination for Convenience by Novartis After the Option Exercise Date. On a Licensed Product-by-Licensed Product basis, in the event that, following the Option Exercise Date, Voyager terminates this Agreement for cause pursuant to Section 10.3.1 or Novartis terminates this Agreement for convenience pursuant to Section 10.5, except as otherwise expressly provided herein, all rights and obligations of each Party hereunder that correspond with such termination will cease (including all rights and licenses and sublicenses granted by either Party to the other Party hereunder corresponding to the terminated Licensed Product(s)); provided that Novartis will have the right, in its sole discretion, to sell any inventory of any Licensed Product affected by such termination that remains on hand as of the effective date of the termination, so long as Novartis pays to Voyager the royalties and other amounts payable hereunder (including milestones) that are applicable to such subsequent sales in the applicable Territory in accordance with the terms of this Agreement.

(c) Termination for Cause by Novartis.

i. Partial Termination. In the event that, on or following the Option Exercise Date, (a) Novartis terminates this Agreement pursuant to Section 10.3.2 or 10.3.3 with respect to a Licensed Product in any country in the Territory, and the event that gave rise to the right of termination materially impairs the ability to Exploit the applicable Licensed Product in the applicable terminated country, then (b) all licenses granted to Novartis under this Agreement with respect to such Licensed Product in such country will become irrevocable and perpetual, and Novartis will have no further obligations to Voyager under this Agreement with respect to such Licensed Product in such country, other than (i) those obligations that expressly survive termination in accordance with Section 10.7.3, an obligation to pay all milestones and royalties under Sections 5.3 and 5.4 with respect to such Licensed Products in such terminated country in an amount equal to [**] percent ([**]%) of the amount that would otherwise have been payable under this Agreement, such amount to be paid in accordance with and subject to the other terms of this Agreement governing such payments, and (iii) in addition to all other rights that it may have at law, Novartis will have the right to offset, against any payment owing to Voyager hereunder, any damages awarded to Novartis in a proceeding under Section 11.3 or agreed by the Parties to be owed by Voyager to Novartis. The foregoing will not be construed to limit Voyager's right to receive the full amount of any payments that accrued before the effective date of such termination.

ii. Complete Termination. In the event that, on or following the Option Exercise Date, (a) Novartis terminates this Agreement in its entirety pursuant to Section 10.3.2 or 10.3.3, and the event that gave rise to the right of termination materially impairs the ability to Exploit the Licensed Products in the United States, then (b) all licenses granted to Novartis under this Agreement with respect to all Licensed Products will become irrevocable and perpetual, and Novartis will have no further obligations to Voyager under this Agreement with respect to such Licensed Products, other than (i) those obligations that expressly survive termination in accordance with Section 10.7.3, (ii) an obligation to pay all milestones and royalties under Sections 5.3 and 5.4 with respect to such Licensed Products in an amount equal to [**] percent ([**]%) of the amount that would otherwise have been payable under this Agreement, such amount to be paid in accordance with and subject to the other terms of this Agreement governing such payments, and (iii) in addition to all other rights that it may have at law, Novartis will have the right to offset, against any payment owing to Voyager hereunder, any damages awarded to Novartis in a proceeding under Section 11.3 or agreed by the Parties to be owed by Voyager to Novartis. The foregoing will not be construed to limit Voyager's right to receive the full amount of any payments that accrued before the effective date of such termination.

10.7.2 Accrued Rights. Termination or expiration of this Agreement for any reason will be without prejudice to any payments that accrued before the effective date of such termination or expiration or rights that will have accrued to the benefit of any Party prior to such termination or expiration, and any and all damages or remedies arising from any breach hereunder. Such termination or expiration will not relieve any Party from obligations which are expressly indicated to survive expiration or termination of this Agreement.

10.7.3 Survival. The provisions of Article 1 (for purposes of interpreting any other surviving provision of this Agreement), Section 3.1.2(b), Article 5 (with respect to payment

obligations accruing prior to expiration or termination and for purposes of calculation and payment of any payment obligations that survive termination under Section 10.7.1(c)), Section 6.1, Section 6.2.4, Section 6.2.5, Section 6.3.5, Section 7.1 through 7.4, Section 7.6, Section 9.1 through 9.5, Section 9.7, Section 10.6 (solely in the event the termination is triggered pursuant to Section 10.6.1(a)), Section 10.7, and Article 11 (excluding Section 11.8), together with any sections that expressly survive (including any perpetual licenses and sublicenses granted hereunder) and remedies for breach of this Agreement, will survive the termination of this Agreement in its entirety or expiration of this Agreement for any reason, in accordance with their respective terms and conditions, and for the duration stated, and where no duration is stated, will survive indefinitely.

ARTICLE 11
MISCELLANEOUS

11.1 Governing Law. This Agreement and any dispute arising from the performance or breach hereof will be governed by and construed and enforced in accordance with the Laws of the Commonwealth of Massachusetts without reference to conflicts of laws principles; provided that with respect to matters involving the enforcement of intellectual property rights, the Laws of the applicable country will apply. The provisions of the United Nations Convention on Contracts for the International Sale of Goods will not apply to this Agreement or any subject matter hereof.

11.2 Dispute Resolution. If a dispute between the Parties arises under this Agreement, either Party will have the right to refer such dispute in writing to the respective Executive Officers, and such Executive Officers will attempt in good faith to resolve such dispute. If the Parties are unable to resolve a given dispute pursuant to this Section 11.2 within [**] after referring such dispute to the Executive Officers, either Party may have the given dispute settled by binding arbitration pursuant to Section 11.3 (subject to the exceptions specified therein).

11.3 Arbitration Request. If a Party intends to begin an arbitration to resolve a dispute arising under this Agreement, such Party will provide written notice (the "Arbitration Request") to the other Party of such intention and a statement of the issues for resolution. From the date of the Arbitration Request and until such time as the dispute has become finally settled, the running of the time periods as to which Party must cure a breach of this Agreement becomes suspended as to any breach that is the subject matter of the dispute.

11.3.1 Additional Issues. Within [**] after the receipt of the Arbitration Request, the other Party may, by written notice, add additional issues for resolution in a statement of counter-issues.

11.3.2 No Arbitration of Patent Issues. Notwithstanding anything to the contrary in this Agreement, any disputes, claims or controversies arising out of, or for which resolution depends in whole or in part on a determination of the ownership, inventorship, interpretation, validity, enforceability, or infringement of United States Patent rights will not be subject to arbitration under this Agreement, but instead may be brought by either Party in the United States District Court for the District of Delaware, before the United States Patent & Trademark Office, before United States appellate courts as applicable.

11.3.3 Arbitration Procedure. Any arbitration pursuant to this Section 11.3 will be held in Boston, Massachusetts unless another location is mutually agreed by the Parties. The arbitration will be governed under the rules of the International Chamber of Commerce, to the exclusion of any inconsistent state Law. The Parties will mutually agree on the rules to govern discovery and the rules of evidence for the arbitration within [**] after the Arbitration Request. If the Parties fail to timely agree to such rules, the United States Federal Rules of Civil Procedure will govern discovery and the United States Federal Rules of Evidence will govern evidence for the arbitration. The arbitration will be conducted by three (3) arbitrators, of which each Party will appoint one, and the arbitrators so appointed will select the third and final arbitrator. The arbitrators will have experience of biotechnology and therapeutics licensing disputes. The arbitrators may proceed to an award, notwithstanding the failure of either Party to participate in the proceedings. The arbitrators will, within [**] after the conclusion of the arbitration hearing, issue a written award and statement of decision describing the essential findings and conclusions on which the award is based, including the calculation of any damages awarded. The arbitrators will be limited in the scope of their authority to resolving only the specific matters which the Parties have referred to arbitration for resolution and will not have authority to render any decision or award on any other issues. Subject to Section 9.7, the arbitrators will be authorized to award compensatory damages, but will not be authorized to award punitive, special, consequential, or any other similar form of damages, or to reform, modify, or materially change this Agreement. The arbitrators also will be authorized to grant any temporary, preliminary or permanent equitable remedy or relief the arbitrators deem just and equitable and within the scope of this Agreement, including an injunction or order for specific performance. The award of the arbitrators will be the sole and exclusive remedy of the Parties, except for those remedies that are set forth in this Agreement or which apply to a Party by operation of the applicable provisions of this Agreement, and the Parties hereby expressly agree to waive the right to appeal from the decisions of the arbitrators, and there will be no appeal to any court or other authority (government or private) from the decision of the arbitrators. Judgment on the award rendered by the arbitrator may be enforced in any court having competent jurisdiction thereof, subject only to revocation of the award on grounds set forth in the United Nations Convention on the Recognition and Enforcement of Foreign Arbitral Awards.

11.3.4 Arbitration Expenses. Each Party will bear its own attorneys' fees, costs, and disbursements arising out of the arbitration, and will pay an equal share of the fees and costs of the arbitrator; provided, however, that the arbitrators, in their award, will be authorized to determine whether a Party is the prevailing Party, and if so, to award to that prevailing Party reimbursement for its reasonable attorneys' fees, costs and disbursements (including, for example, expert witness fees and expenses, transcripts, photocopy charges and travel expenses).

11.3.5 Preliminary Injunctions. Notwithstanding anything in this Agreement to the contrary, a Party may seek any remedy at law or in equity, including the issuance of a temporary restraining order or a preliminary, temporary, or permanent injunction from any court of competent jurisdiction in order to preserve or enforce its rights under this Agreement or to prevent immediate and irreparable injury, loss, or damage on a provisional basis, pending the award of the arbitrator on the ultimate merits of any dispute.

11.3.6 Confidentiality. All proceedings and decisions of the arbitrator will be deemed Confidential Information of each of the Parties and will be subject to Article 7. For clarity,

no information concerning an arbitration, beyond the names of the Parties and the relief requested, may be unilaterally disclosed to a Third Party by any Party unless required by law. In addition, any documentary or other evidence given by a Party or witness in the arbitration shall be treated as Confidential Information by any Party whose access to such evidence arises exclusively as a result of its participation in the arbitration, and shall not be disclosed to any Third Party (other than a witness or expert), except as may be required by applicable Law.

11.4 Assignment. This Agreement may not be assigned or otherwise transferred, nor may any right, interest, or obligation hereunder be assigned or transferred, by either Party without the written consent of the other Party; provided, however, that either Party may assign this Agreement or its rights and obligations under this Agreement to (a) a Third Party that acquires all or substantially all of the business or assets of such Party to which this Agreement relates (whether by merger, reorganization, acquisition, sale or otherwise), and agrees in writing to be bound by the terms of this Agreement, or (b) an Affiliate; provided that in each case of (a) and (b) the assignee will expressly agree to be bound by such Party's obligations under this Agreement and that such Party will remain liable for all of its rights and obligations under this Agreement. Any purported assignment in violation of this Section 11.4 will be void. All terms of this Agreement will remain in full force and effect in the event of a Change of Control of either Party and will be binding upon any Acquiring Entity of either Party. In addition, Novartis may assign its rights and obligations under this Agreement to a Third Party where Novartis or its Affiliate is required or makes a good-faith determination based on advice of legal counsel, to divest a Product in order to comply with Law or the order of any Governmental Authority as a result of a merger or acquisition, provided that the assignee will expressly agree to be bound by Novartis's obligations under this Agreement. Each Party will promptly notify the other Party of any assignment or transfer under the provisions of this Section 11.4.

11.5 Performance by Affiliates and Sublicensees. Each Party may perform some or all of its obligations or exercise some or all of its rights under this Agreement through Affiliates or Sublicensees; provided that each Party hereby acknowledges and agrees that it will be responsible for the full and timely performance and observance of all the covenants, terms, conditions and agreements set forth in this Agreement by its Affiliate(s) and Sublicensees.

11.6 Force Majeure. No Party will be held liable or responsible to the other Party nor be deemed to be in default under, or in breach of any provision of, this Agreement for failure or delay in fulfilling or performing any obligation (other than a payment obligation) of this Agreement when such failure or delay is due to force majeure, whether or not foreseeable as of the Effective Date or thereafter. For purposes of this Agreement, force majeure is defined as any cause beyond the control of the affected Party and without the fault or negligence of such Party, which may include acts of God; material changes in Law; war; civil commotion; destruction of production facilities or materials by fire, flood, earthquake, explosion or storm; labor disturbances; epidemics, pandemics, and the spread of infectious diseases, including COVID-19 (as defined by the World Health Organization and any of the strains, variants, or mutations thereof); quarantines; and failure of public utilities or common carriers. In such event the Party affected by such force majeure will immediately notify the other Party of such inability and a good faith estimate of the period for which such inability is expected to continue based on currently available information. The Party giving such notice will thereupon be excused from such of its obligations under this Agreement as

it is thereby disabled from performing for so long as the condition constituting force majeure continues and the non-performing Party takes Commercially Reasonable Efforts to remove the condition, after which time the Parties will promptly meet to discuss in good faith how to best proceed in a manner consistent with this Agreement. To the extent possible, each Party will use reasonable efforts to minimize the duration of any force majeure.

11.7 Notices. Any notice or request required or permitted to be given under or in connection with this Agreement will be deemed to have been sufficiently given if in writing and personally delivered or sent by certified mail (return receipt requested), or overnight express courier service (signature required), prepaid, to the Party for which such notice is intended, at the address set forth for such Party below:

If to Voyager,

addressed to: Voyager Therapeutics, Inc.
64 Sidney Street, Cambridge, MA 02139
Attention: Chief Executive Officer
Telephone: 857-259-5340

with a copy to (which will not constitute notice):

Voyager Therapeutics, Inc.
64 Sidney Street, Cambridge, MA 02139
Attention: General Counsel
Telephone: 857-259-5340

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Brian A. Johnson, Esq.
Telephone: 617-526-6706
Email: brian.johnson@wilmerhale.com

and an email copy to (which will not constitute notice): Voyager's Alliance Manager, to the contact information provided in accordance with Section 2.1.

If to Novartis,

addressed to:

NovartisPharma AG
Lichtstrasse 35
CH-4056 Basel
Switzerland
Attn: Head of NIBR General Legal, Europe
And an email copy to: [**]

with a copy to (which will not constitute notice):

Novartis Institutes for BioMedical Research, Inc.
250 Massachusetts Avenue
Cambridge, MA 02139 USA
Attn: General Counsel

and an email copy to (which will not constitute notice): Novartis's Alliance Manager, to the contact information provided in accordance with Section 2.1.

Copies of notices may be provided to such other address for such Party as it will have specified by like notice to the other Party, provided that notices of a change of address will be effective only upon receipt thereof. If delivered personally, the date of delivery will be deemed to be the date on which such notice or request was given. If sent by overnight express courier service, the date of delivery will be deemed to be the next Business Day after such notice or request was deposited with such service. If sent by certified mail, the date of delivery will be deemed to be the third (3rd) Business Day after such notice or request was deposited with the U.S. Postal Service.

11.8 Global Trade Control Laws. The Parties acknowledge that certain activities covered by or performed under this Agreement may be subject to laws, regulations, or orders regarding economic sanctions, import controls, or export controls ("Global Trade Control Laws"). Each of the Parties will perform all activities under this Agreement in compliance with all applicable Global Trade Control Laws. Furthermore, with respect to the activities performed under this Agreement, each of the Parties represents, warrants, and covenants that:

11.8.1 It will not, for activities under this Agreement, (i) engage in any such activities in a Restricted Market; (ii) involve individuals ordinarily resident in a Restricted Market; or (iii) include companies, organizations or Governmental Entities from or located in a Restricted Market. "Restricted Market" for purposes of this Agreement means the Crimean Peninsula, Cuba, the Donbass Region, Iran, North Korea, Sudan, and Syria, or any other country or region sanctioned by the United States or European Union.

11.8.2 It is not a Restricted Party and is not owned or controlled by a Restricted Party. With respect to activities performed under this Agreement, neither Party will engage or delegate to any Restricted Party for any activities under this Agreement. Each Party will screen all relevant third parties involved by such Party in the activities under this Agreement under the relevant Restricted Party Lists. "Restricted Party" for purposes of this Agreement means any individual or entity on any of the following "Restricted Party Lists": the list of sanctioned entities maintained by the United Nations; the Specially Designated Nationals List and the Sectoral Sanctions Identifications List of the U.S. Treasury Department's Office of Foreign Assets Control; the U.S. Denied Persons List, the U.S. Entity List, and the U.S. Unverified List of the U.S. Department of Commerce; entities subject to restrictive measures and the Consolidated List of Persons, Groups and Entities Subject to E.U. Financial Sanctions, as implemented by the E.U. Common Foreign & Security Policy; the List of Excluded Individuals / Entities published by the U.S. Health and Human Services' Office of Inspector General; any lists of prohibited or debarred parties established under the U.S. Federal Food Drug and Cosmetic Act; the list of parties

suspended or debarred from contracting with the U.S. government; and similar lists of restricted parties maintained by the Governmental Authorities of the countries that have jurisdiction over the activities conducted under this Agreement.

11.8.3 It will not knowingly transfer to the other Party any goods, software, technology or services that are (i) controlled under the U.S. International Traffic in Arms Regulations or at a level other than EAR99 under the U.S. Export Administration Regulations; or (ii) specifically identified as an E.U. Dual Use Item or on an applicable export control list of another country.

11.9 Waiver. No provision of this Agreement will be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The failure of either Party to assert a right hereunder or to insist upon compliance with any term of this Agreement will not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either Party of any condition or term in any one or more instances will be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

11.10 Severability. If any provision hereof should be invalid, illegal or unenforceable in any jurisdiction, the Parties will negotiate in good faith a valid, legal and enforceable substitute provision that most nearly reflects the original intent of the Parties and all other provisions hereof will remain in full force and effect in such jurisdiction and will be construed in order to maintain this Agreement's existence, validity and enforceability to the greatest extent possible and to carry out the intentions of the Parties as nearly as may be possible. Such invalidity, illegality or unenforceability will not affect the validity, legality or enforceability of such provision in any other jurisdiction.

11.11 Entire Agreement. This Agreement, together with the Schedules and Exhibits hereto, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and sets forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the Parties and supersede and terminate all prior agreements negotiations, correspondence, agreements, and understanding, whether oral or written, between the Parties. In particular, and without limiting the foregoing, this Agreement supersedes and replaces the Existing Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties prior to the Effective Date. No subsequent alteration, amendment, change or addition to this Agreement will be valid or effective unless reduced to writing and signed by the respective authorized officers of each Party.

11.12 Independent Contractors. Nothing herein will be construed to create any relationship of employer and employee, agent and principal, partnership or joint venture or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party between the Parties. Each Party is an independent contractor under this Agreement. Neither Party will have the authority to enter into any contracts or commitments or to incur any

liabilities in the name of, or on behalf of, the other Party, or to bind or obligate the other Party and neither Party will represent that it has such authority.

11.13 Headings; Construction; Interpretation. Headings and any table of contents used herein are for convenience only and will not in any way affect the construction of or be taken into consideration in interpreting this Agreement. The terms of this Agreement represent the results of negotiations between the Parties and their Representatives, each of which has been represented by counsel of its own choosing, and neither of which has acted under duress or compulsion, whether legal, economic or otherwise. Accordingly, the terms of this Agreement will be interpreted and construed in accordance with their usual and customary meanings, and each of the Parties hereby waives the application in connection with the interpretation and construction of this Agreement of any rule of Law to the effect that ambiguous or conflicting terms or provisions contained in this Agreement will be interpreted or construed against the Party whose attorney prepared the executed draft or any earlier draft of this Agreement. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause, Exhibit or Schedule will be deemed to be a reference to any Article, Section, subsection, paragraph, clause, Exhibit or Schedule, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (a) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (b) any reference to any Law refers to such Law includes all rules and regulations thereunder and any successor Law, in each case as from time to time enacted, repealed or amended, (c) the words “herein,” “hereof” and “hereunder,” and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof, (d) the words “include,” “includes,” “including,” “exclude,” “excludes,” and “excluding,” will be deemed to be followed by the phrase “but not limited to,” “without limitation” or words of similar import, (e) the word “or” is used in the inclusive sense (and/or), (f) words in the singular or plural form include the plural and singular form, respectively, (g) references to any gender refer to each other gender, (h) references to a particular Person include such Person’s successors and assigns to the extent not prohibited by this Agreement, and (i) a capitalized term not defined herein but reflecting a different part of speech than a capitalized term which is defined herein will be interpreted in a correlative manner.

11.14 Further Actions. Each Party will execute, acknowledge and deliver such further instruments, and do all such other acts, as may be necessary or appropriate in order to carry out the expressly stated purposes and the clear intent of this Agreement.

11.15 Parties in Interest. All of the terms and provisions of this Agreement will be binding upon, and will inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

11.16 Counterparts. This Agreement may be signed in counterparts, each and every one of which will be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF will be treated as original signatures.

[Signature page follows.]

IN WITNESS WHEREOF, and intending to be legally bound hereby, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

Voyager Therapeutics, Inc.

By: /s/ Michael J. Higgins
Name: Michael J. Higgins
Title: Interim CEO & Chairman of the Board

Novartis Pharma AG

By: /s/ Simone Pfirter
Name: Simone Pfirter
Title: Head NIBR General Legal Europe

Novartis Pharma AG

By: /s/ Petra Grohmann
Name: Petra Grohmann
Title: Authorized Signatory

Exhibit 10.43

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

DATED 3 NOVEMBER 2022

PATENT AND KNOW-HOW LICENCE

BETWEEN

TOUCHLIGHT IP LIMITED

AND

VOYAGER THERAPEUTICS, INC.

This agreement is dated 3 November 2022

Parties

(1) **TOUCHLIGHT IP LIMITED**, a company incorporated under the laws of England and Wales with Company Number 09272417 which has its offices at Morelands and Riverdale Buildings, Lower Sunbury Road, Hampton, TW12 2ER, UK (**"Licensor" or "Touchlight"**)

(2) **VOYAGER THERAPEUTICS, INC.,** incorporated under the laws of Delaware, U.S. with offices at 64 Sidney St, Cambridge, MA 02139, USA (**"Licensee" or "Voyager"**)

BACKGROUND

The Licensor has agreed to grant, and the Licensee has agreed to take, a licence of certain patent rights and know-how on the terms set out in this agreement (the "**Agreement**").

Agreed terms

1. Interpretation

The definitions and rules of interpretation in this clause apply in this Agreement. Other definitions have the respective meanings set forth in this Agreement.

Affiliates	In relation to a party, means any entity or person that Controls, is Controlled by, or is under common Control with that party.
Agreed Communication	The wording set out in the attached Schedule 1.
Agreement	This Patent and Know-How Licence.
Bankruptcy Code	11 U.S.C §§ 101-1330, as amended.
Business Day	Any day other than a Saturday, Sunday or public holiday in England or New York City, New York,

	United States when banks in London and New York are open for business.
Capsid Library	Any library of DNA sequences containing a diversity of variant sequences for the production of adeno-associated virus (AAV) capsids with novel properties .
Confidential Information	Any and all of the following, in whole or in part: (a) information, including technical, scientific, or commercial information that, if provided in documentary form or by way of a model or in other tangible form, at the time of provision is marked or otherwise designated to show expressly or by necessary implication that it is imparted in confidence; (b) information, including technical, scientific, or commercial information that, if imparted orally or by demonstration, at the time of disclosure the disclosing party or its representatives informed the receiving party was imparted in confidence or by necessary implication it is imparted in confidence; (c) any sample or article incorporating or derived from the foregoing information and whether or not provided by the disclosing party; (d) any report provided by Licensee in accordance with Clause 6.9, without regard to whether there is any confidentiality marking or other designation of confidentiality;

	(e) any information regarding any licence or other agreement of Licensee or any Affiliate of Licensee, without regard to whether there is any confidentiality marking or other designation of confidentiality; (f) any note or record of any of the foregoing information imparted orally; (g) the terms of this Agreement; and/or (h) any copy, notes or analyses of any of the foregoing made by the receiving party. For the purposes of this agreement "Confidential Information" shall include any confidential information disclosed under the confidentiality agreement between the parties dated [**].
Control	"Control" (including, with correlative meanings, the terms "controlled by" and "under common control with") as used with respect to a Person, means the possession, directly or indirectly, of the power to direct, or cause the direction of, the management or policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and "control" will be presumed to exist if in the case of a corporate entity, direct or indirect beneficial ownership of 50 per cent (or, outside a party's home territory, such lesser percentage as is the maximum permitted level of foreign investment) or more of the share capital, stock or other participating interest carrying the right to vote

	or to distribution of profits of that party, as the case may be.
dbDNA Library Constructed Capsid	Any capsid created by Voyager during the Licence Term utilizing the Licensed Technology for construction of the corresponding Capsid Library.
Effective Date:	2 May 2017.
Execution Date:	The date of the last signature set forth below.
Field of Use	The application of the Licensed Technology in relation to: (a) the construction of Capsid Libraries; and/or (b) the development and exploitation of capsids selected from such duly constructed Capsid Libraries.
Know-How	Any and all tangible, confidential, proprietary research, technical or scientific information existing before, on or after the Effective Date that is not available to the public.
Licensed Know-How	Any Know-How disclosed or made available by Licensor or any of its Affiliates to Licensee or any of its Affiliates on or after the Effective Date that directly or indirectly relates to manufacture of dbDNA Library Constructed Capsids, including that disclosed to Licensee under a confidentiality agreement dated [**].
Licensed Technology	Techniques for utilizing a linear, double-stranded, covalently-closed DNA molecule flanked at each end by protelomerase binding sequences or parts thereof as recited in the Patent and/or referencing

	or otherwise based upon any aspect of the Licensed Know-How.
Licence Term	The period of time starting on 2 May 2017 and ending on 31 December 2021 inclusive.
Net Sales	The actual gross receipts from sales of a Voyager Therapeutic Product by Licensee, its Affiliates, or therapeutic program collaborators or licensees worldwide, in arm's-length transactions with Third Parties, after the deduction of: a) bad debts actually written off during the accounting period; b) sales returns and allowances actually paid, granted or accrued, including trade, quantity and cash discounts and any other adjustments granted on account of price adjustments, billing errors, rejected goods, damaged or defective goods, recalls, returns, rebates, reimbursements or similar payments granted or given to wholesalers or other distributors, buying groups, health care insurance carriers, chain pharmacies, mass merchandisers, staff model HMOs, pharmacy benefit managers or other institutions;

	c) adjustments arising from consumer discount programs or other similar programs; d) taxes imposed on the production, sale, import, delivery or use of the Voyager Therapeutic Product (including customs or excise duties, sales tax, consumption tax, VAT and other taxes or duties relating to sales, but excluding income taxes); e) compulsory or negotiated payments and cash rebates in respect of sales to the United States government, any state government or any foreign government, or to any other governmental authority, or with respect to any government-subsidized program or managed care organization; and/or f) freight (including storage, shipping and handling) and insurance (to the extent that Licensee or its Affiliates bear the cost of freight including storage, shipping and handling) and insurance. Notwithstanding the foregoing, (x) in the event a Voyager Therapeutic Product is sold by a Voyager licensee of such product, the net sales may (subject to the following) be determined in

	accordance with the operative agreement pursuant to which such Voyager Therapeutic Product is licensed and sold by such Voyager licensee, and Voyager will use commercially reasonable efforts to cause such operative agreements to characterise any payments or costs in accordance with GAAP; and (y) intra-company transfers or intra-company sales by the Licensee conducted in the ordinary course and conduct of the Licensee's activities without specific reference to the application of this Agreement shall not be considered an arm's-length transaction. The assessment of actual gross receipts from sales and quantum of any deductions will be determined from books and records maintained in accordance with GAAP.
Net Receipts	The actual total monies or other financial benefits received directly or indirectly by Licensee and/or its Affiliates under a Non-Therapeutic Program Licence in connection with the research, development, manufacture and commercialisation of Third Party Non-Therapeutic Program Licensed Products including: i. Upfront licence fees (excluding upfront fees for option or access fees paid for the right to exercise an option to license capsids from a Capsid Library, which are addressed separately below) in respect of any dbDNA Library Constructed Capsid. Where a licence is to one or more dbDNA Library Constructed Capsids and in the

	same licence or associated agreement additionally to one or more non-dbDNA Library Constructed Capsids, such fees shall be pro-rata the ratio of dbDNA to non-dbDNA Library Constructed Capsids; ii. Option exercise fees for licences to dbDNA Library Constructed Capsids; iii. Development, regulatory, clinical, or commercial milestone fees achieved in connection with the use of a dbDNA Library Constructed Capsid in a Third Party Non-Therapeutic Program Licensed Product; iv. Royalties including tiered royalties based on Net Sales of the Third Party's sales of their Non-Therapeutic Program Licensed Products; and/or v. the value of any other financial benefit received by Licensee and its Affiliates (as calculated by Licensee), including but not limited to (1) shares, options or other securities; (2) the amount by which any premium received for shares, options or other securities exceeds fair market value; and (3) any loan guarantee or other financial benefit made or given other than on arms' length and market terms.
Upfront Capsid Licence Option Fees	The actual upfront total monies or other financial benefits received in consideration of option rights to Non-Therapeutic Program Licences (i.e., option

	or access rights that would allow for exercise of a license to capsids, one or more of which is a dbDNA Library Constructed Capsid) in connection with the research, development, manufacture and commercialisation of Third Party Non-Therapeutic Program Licensed Products, but excluding any such financial benefits in consideration of such option rights received to date under Voyager's Agreement with [**] and Voyager's Agreement with [**]. Notwithstanding the foregoing, "Upfront Capsid License Option Fees" shall be less: (i) any pre-paid research or development fees defined and quantified as compensation for work to be undertaken by Licensee prior to or subsequent to the payment of licence option fees, (ii) other pre-payment in respect of services and materials of Licensee in carrying out obligations under the applicable agreement or (iii) any payment of prior patent costs, in each case ((i) through (iii)) determined by Voyager acting in good faith and where such sums are included in and expressly specified as part of the upfront fees. By way of example, if Voyager receives an upfront payment of $[**] and such sum includes a stated $[**] by way of pre-paid R&D fees then the Upfront Capsid Licence Option Fees for that agreement shall be $[**].
Patent	[**] and all continuations, continuations-in-part, divisions, extensions, substitutions, reissues, re-examinations and renewals of the same as well as

	any patent or application in the Territory that shares a priority claim with [**].
Person	Any individual, partnership, limited liability company, firm, corporation, association, trust, unincorporated organization or other similar entity or organization.
Territory	United States of America.
Third Party	Any person other than Licensor, Licensee or their respective Affiliates.
Non-Therapeutic Program Licence	A licence through which Voyager grants rights to any Third Party to use a dbDNA Library Constructed Capsid in connection with a product developed by such Third Party other than a Voyager Therapeutic Product.
Non-Therapeutic Program Licensees	Any Third Party to which Voyager grants a Non-Therapeutic Program Licence.
Third Party Non-Therapeutic Program Licensed Products	Any AAV-based product, other than a Voyager Therapeutic Product, developed by a Non-Therapeutic Program Licensee incorporating a dbDNA Library Constructed Capsid.
Voyager Therapeutic Product(s)	Any AAV-based gene therapy product developed by Voyager or its therapeutic program collaborators or therapeutic program licensees, incorporating a dbDNA Library Constructed Capsid.
Voyager's Agreement with [**]	The Option and License Agreement dated [**] between Voyager and [**] in relation to [**].
Voyager's Agreement with [**]	The Option and License Agreement dated [**] between Voyager and [**] for [**].

1.2 Clause and Schedule headings shall not affect the interpretation of this Agreement.

1.3 The Schedules form part of this Agreement and shall have effect as if set out in full in the body of this agreement. Any reference to this Agreement includes the Schedules.

1.4 References to clauses and Schedules are to the clauses and Schedules of this Agreement.

1.5 Unless the context otherwise requires, words in the singular include the plural and in the plural include the singular. Unless the context otherwise requires, a reference to one gender shall include a reference to the other genders.

1.6 Any words following the terms including, include, in particular or any similar expression shall be construed as illustrative and shall not limit the sense of the words preceding those terms.

1.7 A person includes a natural person, corporate or unincorporated body (whether or not having separate legal personality) and that person's legal and personal representatives, successors and permitted assigns.

2. Grant of Licence

2.1 The Licensor hereby grants to the Licensee, a non-exclusive licence for the Licence Term within the Territory, to the Licensed Technology in the Field of Use, to create one or more Capsid Libraries, including any step, method or other process that is necessary therefor or a part thereof. The foregoing limitation to the Territory shall not limit any use or exploitation of any dbDNA Library Constructed Capsid, anywhere in the world.

(a) The Licensee may, at any time, select one or more dbDNA Library Constructed Capsids for use by Licensee, its Affiliates, collaborators or licensees to research, develop, use, sell, supply or otherwise exploit anywhere in the world in connection with one or more:

(i) Third Party Non-Therapeutic Program Licensed Products, and

(ii) Voyager Therapeutic Products.

2.2 Notwithstanding the provisions of clause 2.1 above, the Licensee is granted no rights hereunder: (a) to grant sub-licences (including the right to subcontract) to the Licensed Technology under this agreement, or (b) to any improvements to the Licensed Technology; except that the foregoing in this clause 2.2 does not limit or otherwise restrict Licensee's ability to license any dbDNA Library Constructed Capsid during or after the Licence Term to Licensee's Affiliates, collaborators, or licensees in accordance with clause 2.1(a) and, during the Term, without derogation from Licensee's payment obligations under clause 6. Save for the rights granted by this agreement, the Licensor retains all other rights in the Licensed Technology.

2.3 To the extent that the Bankruptcy Code is applicable, all rights and licenses granted under or pursuant to this Agreement are, and shall otherwise be, deemed to be, for purposes of Section 365(n) of the Bankruptcy Code, licenses of rights to "intellectual property" as defined under Section 101(35A) of the Bankruptcy Code. The Parties shall retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code.

2.4 The Licensee represents that since end of the Licence Term until the date of this Agreement it has not made any use (whether independently or in conjunction with any Third Party) of the Licensed Technology or the processes covered in the Patent anywhere in the world.

2.5 The Licensee undertakes that with effect from the date of this Agreement it shall not:

(a) make any use of the Licensed Technology or the processes covered in the Patent anywhere in the world except for the purposes authorized in this Agreement.

(b) directly or indirectly challenge the validity or enforceability of the Patent in any court proceeding, before the United States Patent and Trademark Office or otherwise, except that nothing herein shall limit Licensee from any direct or indirect challenge of the validity or enforceability of the Patent solely in connection with any actual or threatened litigation asserting the Patent against Licensee, or any Affiliate, collaborator, licensee, customer, contractor or manufacturer of Licensee (other than litigation based solely on Licensor's contract claims under this Agreement); or

(c) voluntarily render aid or counsel, directly or indirectly, any Third Party in connection with challenging the validity of the Patent, except that nothing herein shall limit Licensee from any direct or indirect challenge of the validity or enforceability of the Patent in connection with any actual or threatened litigation asserting the Patent against Licensee, any Affiliate, or Non-Therapeutic Program Licensee or licensee of a Voyager Therapeutic Product in connection with a Non-Therapeutic Program Licence or license of a Voyager Therapeutic Product.

3. Representations and Warranties

3.1 Each party represents and warrants to the other party as of the Execution Date that:

(a) it is a corporation duly organized and validly existing under the laws of the jurisdiction of its organization, and has the full right, power and authority to enter into this agreement and to perform its obligations hereunder;

(b) this agreement has been duly executed by it and is legally binding upon it, enforceable in accordance with its terms, and does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, nor violate any material law or regulation of any court, governmental body or administrative or other agency having jurisdiction over it; and

(c) the execution and delivery of this agreement and the performance of such Party's obligations hereunder (i) do not conflict with or violate any requirement of applicable laws or any provision of the charter, articles of incorporation, bylaws, limited partnership agreement, or any similar instrument of such party, as applicable, in any material way, and (ii) do not conflict with, violate, or breach or constitute a default or require any consent under, any applicable laws or any contractual obligation or court or administrative order by which such party is bound.

3.2 Licensor represents and warrants to Licensee that:

(a) Licensor has all rights necessary to grant to Licensee all rights and licences set forth in this agreement, and Licensor has not granted any right or licence in a

manner that conflicts with its ability to grant any rights or licences to Licensee or limits the scope of any of the rights or licences granted to Licensee; and

(b) Licensor has no patents or patent applications, other than the Patent, that claim or otherwise cover any dbDNA Library Constructed Capsid or its use.

4. Indemnity

4.1 By the Licensee. The Licensee shall indemnify the Licensor from and against all liabilities, costs, expenses, damages and losses, including any legal and other reasonable professional costs and expenses (collectively, "**Losses**") suffered or incurred by the Licensor as a result of a Third Party suit, claim, action, proceeding, hearing, investigation, judgment, order decree, stipulation, injunction or demand ("**Third Party Claim**"), arising out of or in connection with the Licensee's, its Affiliates', collaborators' or licensees' research, development, use, sale, supply or other exploitation of any Third Party Non-Therapeutic Program Licensed Product or any Voyager Therapeutic Product, as applicable, including any Third Party claim of infringement of any intellectual property rights or rights of a similar nature anywhere in the world.

4.2 By the Licensor. The Licensor shall indemnify the Licensee from and against all Losses suffered or incurred by the Licensee as a result of a Third Party Claim arising out of or in connection with any breach of any of Licensor's representations or warranties.

4.3 Conditions. Any party seeking indemnification hereunder must promptly notify the indemnifying party after receipt of actual notice of any Third Party Claim for which it seeks indemnification; *provided*, *however*, that any such failure shall not relieve the indemnifying party of its obligations hereunder except to the extent that the indemnifying party is actually prejudiced by such failure to notify. The indemnifying party shall have sole control and authority with respect to the defense, litigation, compromise or settlement of such claim (except to the extent that any settlement involves material commitments, responsibilities or obligations on the part of the indemnified party, in which case such settlement shall require the prior written consent of the indemnified party, which consent shall not be unreasonably delayed, conditioned or withheld). The indemnifying party shall not be responsible for any settlement it does not approve in writing. The indemnified party shall

provide reasonable information, cooperation and assistance as required by the indemnifying party (at the indemnifying party's expense). The indemnified party reserves the right to participate at its own cost in any proceedings with counsel of its own choosing; *provided*, *however*, that the indemnified party shall at all times be subject to the indemnifying party's sole control and authority with respect to defending, litigating or settling the claim.

5. Confidentiality

5.1 By Licensee. At all times, the Licensee shall keep secret and confidential any Confidential Information of Licensor and shall not: (a) use such Confidential Information for any purpose except for the purpose of exercising or performing its rights and obligations under this agreement; or (b) disclose such Confidential Information to any Third Party other than any of its officers or employees directly concerned in the development, manufacture, use or sale of the Voyager Therapeutic Products or any Third Party Non-Therapeutic Program Licensed Product, or its existing or prospective outside consultants, contractors, investors, collaboration partners, licensees, or professional advisors; provided that, before disclosure to any such Third Party, Licensee shall inform such Third Party of the confidential nature of the information and shall remain responsible for such Third Party's compliance with the confidentiality obligations set out in this clause 5.1.

5.2 By Licensor. At all times, the Licensor shall keep secret and confidential any Confidential Information of Licensee and shall not; (a) use such Confidential Information for any purpose except for the purpose of exercising or performing its rights and obligations under this agreement; and (b) disclose such Confidential Information to any person other than any of its officers or employees directly or indirectly concerned in the licensing of the Patent or its professional advisers; provided that, before disclosure to any such Third Party, Licensor shall inform such Third Party of the confidential nature of the information and is responsible for such Third Party's compliance with the confidentiality obligations set out in this clause 5.2.

5.3 Exceptions. The provisions of this clause 5 shall not apply to Confidential Information that:

(a) was known or available on a non-confidential basis to the receiving party before it was disclosed to it by the disclosing party;

(b) is or becomes generally available to the public (otherwise than through a breach of this clause 5);

(c) was independently developed by the receiving party without reference to any Confidential Information of the disclosing party;

(d) is disclosed to a third party by the disclosing party without any confidentiality restrictions; or

(e) the parties agree in writing is not confidential or may be disclosed.

5.4 Authorized Disclosure. Notwithstanding clauses 5.1 and 5.2, each party may disclose Confidential Information, including the terms of this Agreement, to the extent such disclosure is required by law, court order or any governmental or regulatory authority (including, for example, any disclosure required by any regulatory authority), as long as, to the extent it is legally permitted to do so, the disclosing party shall give the other party as much notice of such disclosure as possible and takes into account the reasonable requests of the other party in relation to the content of such disclosure. Without limiting the foregoing, the existence and terms of this Agreement may be disclosed to the extent required to comply with applicable laws or any self-regulatory organization, including the rules or regulations of the United States Securities and Exchange Commission.

5.5 Agreed Communication. Licensor shall issue the Agreed Communication not less than [**] after the Execution Date.

6. Payments

6.1 Within [**] of execution of this Agreement and in consideration of the rights granted hereunder, the Licensee shall pay to the Licensor the sum of USD 5,000,000 (5 million US dollars) by way of an upfront non-refundable technology access fee, and such sum shall not be returnable nor available for credit against royalties or any other sums payable by the Licensee under this Agreement.

6.2 For payments received from [**] under Voyager's Agreement with [**], the Licensee shall pay to the Licensor the following payments within [**] of the following milestone events:

Milestone Payment	Milestone event
$[**] US dollars)	[**]

6.3 For payments received from Third Parties during the Term in relation to a Non-Therapeutic Program License and a dbDNA Library Constructed Capsid, the Licensee shall pay to the Licensor the following payments within [**] following the end of the quarter in which the applicable fees are received by Voyager from the applicable Non-Therapeutic Program Licensee:

Royalty Payment	Royalty Base
[**]% ([**] percent) of the Net Receipts	Per dbDNA Library Constructed Capsid licensed to [**] under Voyager's Agreement with [**] (for the avoidance of doubt, this is in addition to the above $[**] milestone payment relating to [**]). Per dbDNA Library Constructed Capsid licensed to [**] under Voyager's Agreement with [**] (for the avoidance of doubt, no payment is due to Licensor in relation to [**] payment of the [**] under Voyager's Agreement with [**]).
[**]% ([**] percent) of the Net Receipts	Per dbDNA Library Constructed Capsid licensed by Voyager to any other Non-Therapeutic Program Licensees.

[**]% ([**] percent) of the Upfront Capsid License Option Fees	Per upfront fee paid in consideration of option rights to license any capsid from a selection of capsids that includes dbDNA Library Constructed Capsids under a Non-Therapeutic Program License.

In relation to **Voyager Therapeutic Products**, the Licensee shall pay to the Licensor a royalty of [**]% ([**] percent) of Net Sales of Voyager Therapeutic Products, that are sold or otherwise supplied by or on behalf of the Licensee or its Affiliates (other than to Affiliates) or Voyager Therapeutic Product licensees during the Term.

In addition, Voyager shall pay the following milestone payments in respect of each Voyager Therapeutic Product achieving such milestone during the Term:

(i) $[**] upon the [**];

(ii) $[**] upon the [**]; and

(iii) $[**] upon the [**].

6.4 Both parties shall abide by any auditor's report obtained under clause 6.10 and shall promptly rectify any overpayment or underpayment (as the case may be).

6.5 Royalties and other sums payable under this agreement are:

(a) non-refundable and non-creditable;

(b) exclusive of VAT (or similar tax), and

(c) shall be paid free and clear of all deductions and withholdings whatsoever, unless the deduction or withholding is required by law, except that if the Licensee is required by law to make a deduction or withholding, the Licensee shall, within [**] of making the deduction or withholding, provide a statement in writing showing the gross amount of the payment, the amount of the sum deducted and the actual amount paid. The Licensee shall use all reasonable endeavors to assist the

Licensor to claim recovery or exemption under any double taxation or similar agreement with respect to any such deduction.

6.6 Royalties and other sums payable under this agreement shall be paid in USD (US dollars) within [**] of the applicable payment date (in each case, being the end of the relevant quarterly period in the case of a royalty or achievement of the Milestone, as applicable) to the credit of the following bank account of the Licensor:

Bank Name:	[**]
Bank Country:	[**]
Bank Account Name:	[**]
Bank Account Number:	[**]
Bank Sort Code	[**]
IBAN Code	[**]
BIC/SWIFT	[**]

6.7 For the purposes of this agreement, the exchange rate used to convert net invoice amounts recorded in currencies other than the U.S. Dollar to U.S. Dollars for the applicable accounting period, the conversion to be performed in a manner consistent with Voyager's normal practices used to prepare its audited financial statements for external reporting purposes, provided that such practices use a customary accepted source of published exchange rates

6.8 If there is any delay in paying any sum due under this agreement by the due date, the Licensee shall pay to the Licensor interest (calculated on a monthly basis) on the overdue payment from the date when such payment was due to the date of actual payment at a rate of [**] percent ([**]%) per annum over the base rate of Bank of England from time to time.

6.9 At the same time as payment of royalties falls due, the Licensee shall submit or cause to be submitted to the Licensor a statement in writing recording the calculation of such royalties payable setting forth:

(a) the Quarterly Period for which the royalties were calculated;

(b) the Net Sales of Voyager Therapeutic Products;

(c) the amount of Net Receipts;

(d) the amount and basis of calculation of royalties due and payable;

(e) where relevant, the rate of exchange used; and

(f) the amount of any withholding or other income taxes deductible or due to be deducted from the amount of royalties due and payable.

6.10 No more than [**] during the Term, Licensor may, upon not less than [**] prior written notice to Licensee, designate an independent internationally-recognized accounting firm selected by Licensor and reasonably acceptable to Licensee for the purpose of verifying the reports provided under clause 6.9. Any such audit will be conducted under appropriate confidentiality provisions for the sole purpose of verifying the accuracy and completeness of all financial, accounting and numerical information and calculations provided under this Agreement. The independent accounting firm will only share the results of the audit, not the underlying records, with Licensor and may only share the results of the audit with Licensor at the same time as shared with Licensee. Any audit conducted hereunder will be paid for by Licensor, except if the results of the audit reveal an underpayment by Licensee of [**] percent ([**]%) or more, in which case the reasonable fees and expenses for such audit will be paid by Licensee. If such audit shows that: (a) the amount paid by the Licensee is less than the amount due, the Licensee shall pay to the Licensor within [**] of the result of such audit and (b) the amount paid to the Licensor is more than the amount due, the Licensor shall pay to the Licensor within [**] of the result of such audit.

6.11 Within [**] of the end of each calendar year during the Term, the Licensee shall submit to the Licensor a written statement that demonstrates on a de-identified basis, without disclosing any sequence information, that all capsids produced using any method or process which falls within the scope of the Patent claims have been included in the royalty or other payments required under this Agreement. Where the Licensor reasonably believes that there is an error in the written statement, the Licensor shall have the right to appoint an external Third Party expert reasonably acceptable to Licensee and bound by

appropriate confidentiality obligations in favor of the Licensee who shall be given access to Licensee's records, on no less than [**] prior notice, to allow such Third Party expert to assess on a de-identified basis the provenance of all relevant and potentially relevant capsids.

7. Assignment and other dealings

7.1 The Licensee shall not without the prior written consent of the Licensor assign, transfer, mortgage, charge or deal in any other manner with any of its rights or obligations under this agreement, except that in the event of a sale of Licensee or sale of all, or substantially all, the business or assets to which this agreement pertains, no consent shall be required from Licensor.

7.2 The Licensor may at any time and without the consent of the Licensee assign, transfer, mortgage, charge or deal in any other manner with any or all of its rights or obligations under this agreement, provided that the Licensor shall provide prompt written notice to Licensee of the foregoing.

8. Duration and termination

8.1 This agreement shall be effective as of the Effective Date and shall remain in force until the earliest to occur of the following events:

(a) 17 January 2031 being the expiration of the Patent; and

(b) a final, unappealable decision by a patent authority or court of competent jurisdiction invalidating or otherwise rendering unenforceable the claims of the Patent in the United States

(the "**Term**").

8.2 Notwithstanding clause 8.1, in respect of any payments under Clause 6.2 and Clause 6.3 above for any Non-Therapeutic Program Licence entered into during the Term, following the end of the Term, Licensee shall remain responsible for payment of [**]% of any amounts that would otherwise be due to Licensor under Clause 6. 2 and Clause 6.3 in relation to such Non-Therapeutic Program Licence as if the Term had not expired.

8.3 Notwithstanding anything to the contrary in this Agreement, the parties agree and acknowledge that no payments shall at any time be due under this Agreement, during or after the Term, in relation to any use of any capsid that is not a dbDNA Library Constructed Capsid.

8.4 The following provisions shall survive expiration of this Agreement: clause 1 (for the purpose of interpreting the Agreement), clause 2.1(a), clause 2.3, clause 4, clause 5, clauses 6.2 through 6.10 (clauses 6.2 through 6.10, solely to the extent necessary for the survival of clause 8.2), 8.2, 8.3, 8.4, 10 through 21.

9. Further assurance

At its own expense each party shall, and shall use all reasonable endeavours to procure that any necessary Third Party shall, promptly execute such documents and perform such acts as may reasonably be required for the purpose of giving full effect to this Agreement.

10. Waiver

No failure or delay by a party to exercise any right or remedy provided under this Agreement or by law shall constitute a waiver of that or any other right or remedy, nor shall it preclude or restrict the further exercise of that or any other right or remedy. No single or partial exercise of such right or remedy shall preclude or restrict the further exercise of that or any other right or remedy.

11. Entire agreement

Except for any instrument executed together with this Agreement concerning the release of potential claims arising from activities related to the Licensed Technology, this Agreement and the documents referred to in it constitute the entire agreement between the parties and supersedes and extinguishes all previous agreements, promises, assurances, warranties, representations and understandings between them, whether written or oral, relating to its subject matter, including the confidentiality agreement between the parties dated [**] and the non-binding term sheet between the parties dated [**].

12. Variation

No variation of this Agreement shall be effective unless it is in writing and signed by the parties (or their authorised representatives).

13. Severance

13.1 If any provision or part-provision of this Agreement is or becomes invalid, illegal or unenforceable, it shall be deemed deleted, but that shall not affect the validity and enforceability of the rest of this Agreement.

13.2 If any provision or part-provision of this Agreement is deemed deleted, the parties shall negotiate in good faith to agree a replacement provision that, to the greatest extent possible, achieves the intended commercial result of the original provision.

14. Counterparts

This Agreement may be executed in any number of counterparts, including facsimile or PDF copies or electronic signatures, each of which shall be deemed to have the same effect as an original signature, and each of which constitute a duplicate original, but all the counterparts shall together constitute the one agreement.

15. Contracts (Rights of Third Parties) Act 1999

The parties agree that the terms of this Agreement are not enforceable by any third party under the Contracts (Rights of Third Parties) Act 1999.

16. No partnership or agency

Nothing in this Agreement is intended to, or shall be deemed to, establish any partnership or joint venture between the parties, constitute either party the agent of the other party, nor authorise either party to make or enter into any commitments for or on behalf of the other party.

17. Force majeure

Neither party shall be in breach of this Agreement nor liable for delay in performing, or failure to perform, any of its obligations under this agreement if such delay or failure result from events, circumstances or causes beyond its reasonable control. In such circumstances the time for performance shall be extended by a period equivalent to the period during which performance of the obligation has been delayed or failed to be performed.

18. Notices

18.1 Any notice required to be given under this Agreement shall be in writing and shall be delivered personally, or sent by prepaid first-class post or recorded delivery or by commercial courier, to each party required to receive the notice at its address as set out below:

(a) Licensor: Touchlight IP Ltd, Morelands and Riverdale Buildings, Lower Sunbury Road, Hampton, TW12 2ER, UK email *[**]*, with a copy (delivery of which shall not constitute valid delivery of notice to each of *[**]* and *[**]*; and

(b) Licensee: Voyager Therapeutics, Inc., 64 Sidney St, Cambridge, MA 02139, Attention: General Counsel, with a copy (delivery of which shall not constitute valid delivery of notice) to Wilmer Cutler Pickering Hale and Dorr LLP, 60 State Street, Boston, MA 02109, Brian A. Johnson, Esq., *[**]*;

or as otherwise specified by the relevant party by notice in writing to each other party.

18.2 Any notice shall be deemed to have been duly received:

(a) if delivered personally, when left at the address and for the contact referred to in this clause;

(b) if sent by prepaid first-class post or recorded delivery, at 9.00 am on the fifth Business Day after posting; or

(c) if delivered by commercial courier, on the date and at the time that the courier's delivery receipt is signed.

18.3 A notice required to be given under this Agreement shall not be validly given if sent by e-mail only.

18.4 The provisions of this clause 18 shall not apply to the service of any proceedings or other documents in any legal action.

19. Dispute resolution procedure

19.1 If a dispute arises out of or in connection with this Agreement or the performance, validity or enforceability of it then, except as expressly provided in this agreement, the parties shall follow the dispute resolution procedure set out in this clause:

(a) The parties will within [**] of a written request from one party to the other, meet in a good faith attempt to resolve the dispute in meetings between representatives of senior management

(b) If resolution has not been reached at that meeting, the parties agree to enter into mediation in good faith to settle such a dispute and will do so in accordance with the CEDR Model Mediation Procedure. Unless otherwise agreed between the parties within [**] of notice of the dispute, the mediator will be nominated by CEDR. To initiate the mediation a party must give notice in writing (**"ADR notice"**) to the other party to the dispute, referring the dispute to mediation. A copy of the request should be sent to CEDR. Unless otherwise agreed, the mediation will start not later than [**] after the date of the ADR notice.

19.2 No party may commence any court proceedings in relation to any dispute arising out of this agreement until it has attempted to settle the dispute by mediation and either the mediation has terminated or the other party has failed to participate in the mediation, provided that the right to issue proceedings is not prejudiced by a delay.

20. Governing law

Subject to the parties following the Dispute Resolution procedure, this Agreement and any dispute or claim arising out of or in connection with it or its subject matter or formation shall be governed by and construed in accordance with the law of England and Wales, except that any actions under

patent law shall be governed by the patent laws of the United States or patent laws of other jurisdictions applicable to activities in such jurisdictions.

21. Jurisdiction

Each party irrevocably agrees that the courts of England and Wales shall have exclusive jurisdiction to settle any dispute or claim arising out of or in connection with this Agreement or its subject matter or formation, except that any action under patent law shall be brought in a federal court of competent jurisdiction in the United States.

This Agreement has been entered into on the date stated at the beginning of it.

For and on behalf of **Touchlight IP Ltd**	For and on behalf of **Voyager Therapeutics, Inc.**
Signed /s/ Jonny Ohlson	**Signed** /s/ Allen Nunnally
Print Name Jonny Ohlson	**Print Name** Allen Nunnally
Job Title Executive Chairman	**Job Title** Chief Business Officer
Date 3 November, 2022	**Date** November 4, 2022

Exhibit 10.44

EXECUTION VERSION

STOCK PURCHASE AGREEMENT

By and Between

NEUROCRINE BIOSCIENCES, INC.

AND

VOYAGER THERAPEUTICS, INC.

Dated as of January 8, 2023

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Exhibit A – Notices

STOCK PURCHASE AGREEMENT

THIS STOCK PURCHASE AGREEMENT (this "**Agreement**"), dated as of January 8, 2023 (the "**Signing Date**"), by and between Neurocrine Biosciences, Inc. (the "**Investor**"), a Delaware corporation with its principal place of business at 12780 El Camino Real, San Diego, CA 92130, and Voyager Therapeutics, Inc. (the "**Company**"), a Delaware corporation with its principal place of business at 64 Sidney Street, Cambridge, MA 02139.

WHEREAS, pursuant to the terms and subject to the conditions set forth in this Agreement, the Company desires to issue and sell to the Investor, and the Investor desires to subscribe for and purchase from the Company, certain shares of common stock, par value $0.001 per share, of the Company (the "**Common Stock**"); and

WHEREAS, simultaneously with the execution of this Agreement, the Company and the Investor are entering into the Collaboration Agreement and the Investor Agreement.

NOW, THEREFORE, in consideration of the following mutual promises and obligations, and for good and valuable consideration, the adequacy and sufficiency of which are hereby acknowledged, the Investor and the Company agree as follows:

1. Definitions.

1.1 Defined Terms. When used in this Agreement, the following terms shall have the respective meanings specified therefor below:

"**2014 Stock Option and Grant Plan**" shall mean the Company's 2014 Stock Option and Grant Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**2015 Employee Stock Purchase Plan**" shall mean the Company's 2015 Employee Stock Purchase Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**2015 Stock Option and Incentive Plan**" shall mean the Company's 2015 Stock Option and Incentive Plan, as amended to date and as the same may be amended and/or restated from time to time.

"**Affiliate**" shall mean, with respect to any Person, another Person that, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such other Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if such Person (i) owns, directly or indirectly, beneficially or legally, more than fifty percent (50%) of the outstanding voting securities or capital stock of such other Person, or has other comparable ownership interest(s) with respect to any Person other than a corporation; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of the

Company's Affiliates, nor shall the Company or any of the Company's Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

"**Aggregate Purchase Price**" shall mean the product of the number of Shares issuable hereunder and the Per-Share Purchase Price.

"**Agreement**" shall have the meaning set forth in the Preamble, including all Exhibits attached hereto.

"**Board**" shall mean the Board of Directors of the Company.

"**Business Day**" shall mean a day on which banking institutions in Boston, Massachusetts, United States and San Diego, California, United States are open for business, excluding any Saturday or Sunday.

"**Closing Conditions**" shall mean the conditions to Closing set forth in Sections 6, 7, and 8 hereof.

"**Collaboration Agreement**" shall mean the Collaboration and License Agreement, of even date herewith, between the Investor and the Company, as the same may be amended and/or restated from time to time.

"**Company Financial Advisors**" shall mean Chestnut Securities, Inc.

"**DOJ**" shall mean the U.S. Department of Justice.

"**Effect**" shall have the meaning set forth in the definition of "Material Adverse Effect."

"**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

"**FTC**" shall mean the U.S. Federal Trade Commission.

"**FTC Act**" shall mean the Federal Trade Commission Act, as amended.

"**GAAP**" shall mean generally accepted accounting principles in the United States.

"**Governmental Authority**" shall mean any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

"**HSR Act**" shall mean the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended from time to time.

"**HSR Clearance**" shall mean the expiration or termination of all applicable waiting periods and requests for information (and any extensions thereof) under the HSR Act.

"**HSR Filing**" shall mean the filings by the Company and the Investor with the FTC and the Antitrust Division of the DOJ of a Notification and Report Form for Certain Mergers and Acquisitions (as that term is defined in the HSR Act) with respect to the matters set forth in the Transaction Agreements and the Collaboration Agreement, together with all required documentary attachments thereto.

"**Investor Agreement**" shall mean that certain Amended and Restated Investor Agreement, of even date herewith, between the Investor and the Company, as the same may be amended and/or restated from time to time.

"LAS" shall mean the Nasdaq Notification Form: Listing of Additional Shares.

"**Law"** shall mean any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

"**Material Adverse Effect**" shall mean any change, event or occurrence (each, an "**Effect**") that, individually or when taken together with all other Effects that have occurred prior to the date of determination of the occurrence of the Material Adverse Event, has had a material adverse effect on the business, properties, management, financial position, stockholders' equity or results of operations of the Company and its subsidiaries taken as a whole or on the performance by the Company of its obligations under the Transaction Agreements, except to the extent that any such Effect results from or arises out of: (A) changes in conditions in the United States or global economy or capital or financial markets generally, including changes in interest or exchange rates, (B) changes in general legal, regulatory, political, economic or business conditions or changes in generally accepted accounting principles in the United States or interpretations thereof, (C) acts of war, sabotage or terrorism, or any escalation or worsening of any such acts of war, sabotage or terrorism, (D) earthquakes, hurricanes, floods or other natural disasters, (E) any epidemic, pandemic, or disease outbreak (including the COVID-19 virus) or any escalation or worsening thereof, (F) the announcement of the Transaction Agreements, the Collaboration Agreement or the Transaction, (G) any change in the Company's stock price or trading volume or any failure to meet internal projections or forecasts or published revenue or earnings projections of industry analysts (provided that the underlying events giving rise to any such change shall not be excluded) or (H) any breach, violation or non-performance by the Investor or any of its Affiliates under the Collaboration Agreement, provided, however, that the Effects excluded in clauses (A), (B), (C), (D) and (E) shall only be excluded to the extent such Effects are not disproportionately adverse on the Company and its subsidiaries as compared to other companies operating in the Company's industry.

"**Per-Share Purchase Price**" shall mean $8.88.

"**Person"** shall mean any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, Governmental Authority or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

"**Rule 144**" shall mean Rule 144 promulgated under the Securities Act.

"**Sales Agreement**" shall mean that certain Sales Agreement, by and between the Company and Cowen and Company, LLC, dated as of November 8, 2022.

"**SEC**" shall mean the U.S. Securities and Exchange Commission.

"**Securities Act**" shall mean the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

"**Termination Date**" shall mean the date that is twelve (12) months after the effective date of the HSR Filing.

"**Third Party"** shall mean any Person other than the Investor, the Company or any Affiliate of the Investor or the Company.

"**Transaction"** shall mean the issuance and sale of the Shares by the Company, and the purchase of the Shares by the Investor, in accordance with the terms hereof.

"**Transaction Agreements"** shall mean this Agreement and the Investor Agreement.

"**Transfer Agent**" shall mean the Company's transfer agent.

1.2 Additional Defined Terms. In addition to the terms defined in Section 1.1 hereof, the following terms shall have the respective meanings assigned thereto in the sections indicated below:

Defined Term	Section
2019 Shares	Section 5.8
Closing	Section 3.1
Closing Date	Section 3.1
Common Stock	Preamble
Company	Preamble
Company SEC Documents	Section 4.11(a)
Investor	Preamble
Modified Clause	Section 11.6
Shares	Section 2.1
Signing Date	Preamble

2. Purchase and Sale of Common Stock.

Subject to the terms and conditions of this Agreement, at the Closing, the Company shall issue and sell to the Investor, and the Investor shall purchase from the Company, 4,395,588 shares of Common Stock (the "**Shares**").

3. Closing Date; Deliveries.

3.1 Closing Date. The closing of the purchase and sale of the Shares hereunder (the "**Closing**") shall take place remotely via the exchange of documents and signatures at 9:00 a.m. New York City time on the second (2nd) Business Day following the satisfaction or waiver of all of the Closing Conditions (other than those conditions that by their nature are to be satisfied at the Closing, but subject to the satisfaction at such time of such conditions), or at such other time, date, and location as the parties may agree. The date the Closing occurs is hereinafter referred to as the "**Closing Date**."

3.2 Deliveries.

(a) Deliveries by the Company. At the Closing, the Company shall deliver, or cause to be delivered, to the Investor the Shares, registered in the name of the Investor, and the Company shall instruct its transfer agent to register such issuance at the time of such issuance. The Company shall also deliver at the Closing: (i) a certificate in form and substance reasonably satisfactory to the Investor and duly executed on behalf of the Company by an authorized executive officer of the Company, certifying that the conditions to Closing set forth in Sections 6 and 8.2 hereof have been fulfilled and (ii) a certificate of the secretary or assistant secretary of the Company dated as of the Closing Date certifying (A) that attached thereto is a true and complete copy of the Amended and Restated By-laws of the Company as in effect at the time of the actions by the Board referred to in clause (B) below and on the Closing Date; (B) that attached thereto is a true and complete copy of all resolutions adopted by the Board authorizing the execution, delivery and performance of the Transaction Agreements and the Transaction and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby and thereby as of the Closing Date; (C) that attached thereto is a true and complete copy of the Company's Fifth Amended and Restated Certificate of Incorporation as in effect at the time of the actions by the Board referred to in clause (B) above and on the Closing Date; and (D) as to the incumbency and specimen signature of any officer of the Company executing a Transaction Agreement on behalf of the Company.

(b) Deliveries by the Investor. At the Closing, the Investor shall deliver, or cause to be delivered, to the Company the Aggregate Purchase Price by wire transfer of immediately available United States funds to an account designated by the Company. The Company shall notify the Investor in writing of the wiring instructions for such account not less than two (2) Business Days before the Closing Date. The Investor shall also deliver, or cause to be delivered, at the Closing: (i) a certificate in form and substance reasonably satisfactory to the Company duly executed by an authorized executive officer of the Investor certifying that the conditions to Closing set forth in Section

7 hereof have been fulfilled and (ii) a certificate of the secretary or assistant secretary of the Investor dated as of the Closing Date certifying as to the incumbency and specimen signature of any officer executing a Transaction Agreement on behalf of the Investor.

4. Representations and Warranties of the Company. The Company hereby represents and warrants to the Investor that:

4.1 Organization, Good Standing and Qualification.

(a) The Company has been duly organized and is validly existing and in good standing under the laws of its jurisdiction of organization, is duly qualified to do business and is in good standing in each jurisdiction in which its ownership or lease of property or the conduct of its businesses requires such qualification, and has all power and authority necessary to own or hold its properties and to conduct the businesses in which it is engaged, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, have a Material Adverse Effect.

(b) The Company has all requisite corporate power and corporate authority to enter into the Transaction Agreements and the Collaboration Agreement, to issue and sell the Shares and to perform its obligations under and to carry out the other transactions contemplated by the Transaction Agreements and the Collaboration Agreement.

4.2 Capitalization and Voting Rights.

(a) As of the Signing Date, the authorized capital of the Company consists of: (i) 120,000,000 shares of Common Stock, of which (A) 38,696,454 shares are issued and outstanding, (B) 7,438,643 shares are issuable upon the exercise of outstanding stock options or upon the settlement of outstanding equity awards issued pursuant to the 2014 Stock Option and Grant Plan, the 2015 Stock Option and Incentive Plan, or inducement awards in accordance with Nasdaq Listing Rule 5635(c)(4), (C) 3,391,532 shares are reserved for future issuance pursuant to the 2015 Stock Option and Incentive Plan, and (D) 1,884,309 shares are reserved for future issuance pursuant to the 2015 Employee Stock Purchase Plan, and (ii) 5,000,000 shares of preferred stock, par value $0.001 per share, of which no shares are issued and outstanding. The Company is also party to the Sales Agreement pursuant to which the Company may issue and sell shares of its Common Stock having an aggregate offering price of up to $75,000,000 through Cowen and Company, LLC, from time to time, in “at-the-market” offerings or certain negotiated transactions. All of the issued and outstanding shares of Common Stock have been duly authorized and validly issued and are fully paid and non-assessable, were issued in compliance with federal and state securities Laws, and are not subject to any pre-emptive rights.

(b) Except as described or referred to in Section 4.2(a) above and as provided in the Investor Agreement, as of the Signing Date, there are no outstanding rights (including, without limitation, pre-emptive rights), warrants or options to acquire, or

instruments convertible into or exchangeable for, any shares of capital stock or other equity interest in the Company, or any contract, commitment, agreement, understanding or arrangement of any kind relating to the issuance of any capital stock of the Company, any such convertible or exchangeable securities or any such rights, warrants or options.

(c) Except as disclosed in the Company SEC Documents, no Person has any right to cause the Company to effect the registration under the Securities Act of any securities of the Company.

(d) The Common Stock is registered pursuant to Section 12(b) or 12(g) of the Exchange Act, and the Company has taken no action designed to terminate, or which to its knowledge is likely to have the effect of terminating, the registration of the Common Stock under the Exchange Act nor has the Company received any notification that the SEC is contemplating terminating such registration.

4.3 Subsidiaries. As of the Signing Date, the Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Schedule 1 hereto. All the outstanding shares of capital stock or other equity interests of each subsidiary owned, directly or indirectly, by the Company have been duly authorized and validly issued, are fully paid and non-assessable (except, in the case of any foreign subsidiary, for directors' qualifying shares) and are owned directly or indirectly by the Company, free and clear of any lien, charge, encumbrance, security interest, restriction on voting or transfer or any other claim of any third party.

4.4 Authorization.

(a) The Company has full right, power and authority to execute and deliver the Transaction Agreements and the Collaboration Agreement and to perform its obligations hereunder and thereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of each of the Transaction Agreements and the Collaboration Agreement and the consummation by it of the transactions contemplated thereby has been duly and validly taken.

(b) The Transaction Agreements and the Collaboration Agreement have been duly executed and delivered by the Company and, upon the due execution and delivery of the Transaction Agreements and the Collaboration Agreement by the Investor, will constitute valid and legally binding obligations of the Company, enforceable against the Company in accordance with their respective terms, except, with respect to the Investor Agreement and the Collaboration Agreement, as enforceability may be limited by applicable bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally or by equitable principles relating to enforceability (collectively, the "**Enforceability Exceptions**").

(c) No stop order or suspension of trading of the Common Stock has been imposed by the Nasdaq Stock Market, the SEC or any other Governmental Authority and remains in effect.

4.5 No Defaults. The Company is not (i) in violation of its charter or by-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company is a party, by which the Company is bound or to which any of the property or assets of the Company is subject; or (iii) in violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority having jurisdiction over the Company or any of its subsidiaries, except, in the case of clauses (ii) and (iii) above, for any such default or violation that would not, individually or in the aggregate, have a Material Adverse Effect.

4.6 No Conflicts. The execution, delivery and performance of the Transaction Agreements and the Collaboration Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by the Transaction Agreements and the Collaboration Agreement will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, or result in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Company pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company is a party, by which the Company is bound or to which any of the property or assets of the Company is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Company or (iii) result in the violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority having jurisdiction over the Company or any of its subsidiaries, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation or default that would not, individually or in the aggregate, have a Material Adverse Effect.

4.7 No Governmental Authority or Third-Party Consents. No consent, approval, authorization, order, license, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Company of each of the Transaction Agreements or the Collaboration Agreement or the issuance and sale of the Shares, except (i) such filings as may be required to be made with the SEC and with any state blue sky or securities regulatory authority, which filings shall be made in a timely manner in accordance with all applicable Laws, (ii) as required pursuant to the HSR Act and (iii) with respect to the Shares, the filing with the Nasdaq Stock Market of, and the absence of unresolved issues with respect to, an LAS and a Nasdaq Shares Outstanding Change Form, in each case to the extent required.

4.8 Valid Issuance of Shares. When issued, sold and delivered at the Closing in accordance with the terms hereof for the Aggregate Purchase Price, the Shares shall be duly authorized, validly issued, fully paid and nonassessable and free from any liens, encumbrances or restrictions on transfer, including pre-emptive rights, rights of first refusal or other similar rights, other than as arising pursuant to the Transaction Agreements, as a result of any action by the Investor or under federal or state securities Laws.

4.9 Litigation. There are no legal, governmental or regulatory investigations, actions, suits or proceedings pending to which the Company is a party or to which any property of the Company is subject that, individually or in the aggregate, would reasonably be expected to have a Material Adverse Effect; and no such investigations, actions, suits or proceedings are, to the knowledge of the Company, threatened or contemplated by any governmental or regulatory authority or others.

4.10 Licenses and Other Rights; Compliance with Laws. The Company and its subsidiaries possess or are in the process of obtaining all licenses, certificates, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in the Company SEC Documents, except where the failure to possess or make the same would not, individually or in the aggregate, have a Material Adverse Effect; and except as described in the Company SEC Documents, neither the Company nor any of its subsidiaries has received notice of any revocation or modification of any such license, certificate, permit or authorization or has any reason to believe that any such license, certificate, permit or authorization will not be renewed. The Company and its subsidiaries are, and at all times since January 1, 2021, have been, in compliance with all statutes, rules and regulations applicable to the ownership, packaging, processing, use, distribution, import, or export of any product manufactured or distributed by the Company or its subsidiaries, except where such noncompliance would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

4.11 Company SEC Documents; Financial Statements; Nasdaq Stock Market.

(a) Since January 1, 2021, the Company has timely filed all required reports, schedules, forms, statements and other documents (including exhibits and all other information incorporated therein) required to be filed by it under the Securities Act and the Exchange Act, and any required amendments to any of the foregoing, with the SEC (the "**Company SEC Documents**"). As of its respective filing date, each of the Company SEC Documents complied in all material respects with the requirements of the Securities Act, the Exchange Act, and the rules and regulations of the SEC promulgated thereunder applicable to such Company SEC Documents, and no Company SEC Documents when filed, declared effective or mailed, as applicable, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(b) As of the Signing Date, there are no outstanding or unresolved comments in comment letters received from the SEC or its staff.

(c) The financial statements of the Company included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2021 and in its quarterly reports on Form 10-Q for the quarterly periods ended March 31, 2022; June 30, 2022; and September 30, 2022 fairly present the financial position of the Company and its

consolidated subsidiaries as of the dates indicated and the results of their operations and the changes in their cash flows for the periods specified; such financial statements have been prepared in conformity with GAAP applied on a consistent basis throughout the periods covered thereby, except as otherwise disclosed therein and, in the case of unaudited, interim financial statements, subject to normal year-end audit adjustments and the exclusion of certain footnotes, and any supporting schedules included in the Company SEC Documents present fairly the information required to be stated therein.

(d) The Common Stock is listed on the Nasdaq Stock Market, and the Company has taken no action designed to, or which is likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act or delisting the Common Stock from the Nasdaq Stock Market. The Company has not received any notification that, and has no knowledge that, the SEC or the Nasdaq Stock Market is contemplating terminating such listing or registration.

(e) The Company and its subsidiaries have established systems of "internal control over financial reporting" (as defined in Rule 13a-15(f) of the Exchange Act) that have been designed by, or under the supervision of, their respective principal executive and principal financial officers, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP, including, but not limited to, internal accounting controls sufficient to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences and (v) interactive data in eXtensible Business Reporting Language included in the Company SEC Documents fairly presents the information called for in all material respects and is prepared in accordance with the SEC's rules and guidelines applicable thereto. Except as disclosed in the Company SEC Documents, there are no material weaknesses in the Company's internal controls. The Company's auditors and the Audit Committee of the Board have been advised of: (i) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which have adversely affected or are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and (ii) any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting.

(f) The Company maintains disclosure controls and procedures (as defined in Rule 13a-15(e) of the Exchange Act) that comply with the requirements of the Exchange Act; such disclosure controls and procedures have been designed to ensure that information required to be disclosed by the Company in reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, including controls and procedures designed to ensure that such information is accumulated and communicated to the Company's

management to allow timely decisions regarding disclosures. The Company has conducted evaluations of the effectiveness of its disclosure controls as required by Rule 13a-15 of the Exchange Act.

(g) There is and has been no material failure on the part of the Company or, to the knowledge of the Company, any of the Company's directors or officers, in their capacities as such, to comply with any applicable provision of the Sarbanes-Oxley Act of 2002 and the rules and regulations promulgated in connection therewith, including Section 402 related to loans and Sections 302 and 906 related to certifications.

4.12 Absence of Certain Changes.

(a) Except as disclosed in the Company SEC Documents, since September 30, 2022, (i) there has not been any material change in the capital stock (other than (x) the issuance of shares of Common Stock upon exercise of stock options, the settlement of equity awards and the exercise of warrants described as outstanding in, and the grant of options and awards under existing equity incentive plans described in, the Company SEC Documents and (y) the issuance of shares of Common Stock, options and equity awards granted to new employees of the Company as inducement awards pursuant to Nasdaq Listing Rule 5635(c)(4)), short-term debt or long-term debt of the Company or any of its subsidiaries, or any dividend or distribution of any kind declared, set aside for payment, paid or made by the Company on any class of capital stock, or any material adverse change, or any development that would reasonably be expected to result in a material adverse change, in or affecting the business, properties, management, financial position, stockholders' equity, results of operations of the Company and its subsidiaries taken as a whole; (ii) neither the Company nor any of its subsidiaries has entered into any transaction or agreement (whether or not in the ordinary course of business) that is material to the Company and its subsidiaries taken as a whole or incurred any liability or obligation, direct or contingent, that is material to the Company and its subsidiaries taken as a whole; and (iii) neither the Company nor any of its subsidiaries has sustained any loss or interference with its business that is material to the Company and its subsidiaries taken as a whole and that is either from fire, explosion, flood or other calamity, whether or not covered by insurance, or from any labor disturbance or dispute or any action, order or decree of any court or arbitrator or governmental or regulatory authority.

4.13 Offering. Subject to the accuracy of the Investor's representations set forth in Sections 5.5, 5.6, 5.7, 5.9, 5.10 and 5.11 hereof, the offer, sale and issuance of the Shares to be issued in conformity with the terms of this Agreement constitute transactions which are exempt from the registration requirements of the Securities Act and from all applicable state registration or qualification requirements. Neither the Company nor any Person acting on its behalf will take any action that would cause the loss of such exemption.

4.14 No Integration. The Company has not, directly or through any agent, sold, offered for sale, solicited offers to buy or otherwise negotiated in respect of, any security (as defined in the Securities Act), that is or will be integrated with the sale of

the Shares in a manner that would require registration of the Shares under the Securities Act.

4.15 Brokers' or Finders' Fees. Except with respect to the Company Financial Advisors, neither the Company nor any of its subsidiaries is a party to any contract, agreement or understanding with any person (other than this Agreement) that would give rise to a valid claim against the Company or any of its subsidiaries for a brokerage commission, finder's fee or like payment in connection with the transactions contemplated by the Transaction Agreements and the Collaboration Agreement.

4.16 Investment Company. The Company is not and, immediately after giving effect to the offering and sale of the Shares and the application of the proceeds thereof, will not be required to register as an "investment company" or an entity "controlled" by an "investment company" within the meaning of the Investment Company Act of 1940, as amended, and the rules and regulations of the SEC thereunder.

4.17 No General Solicitation. Neither the Company nor any person acting on behalf of the Company has offered or sold any of the Shares by any form of general solicitation or general advertising. The Company has offered the Shares for sale only to the Investor.

4.18 Foreign Corrupt Practices. Neither the Company nor, to the knowledge of the Company, any agent or other person acting on behalf of the Company, has: (i) directly or indirectly used any funds for unlawful contributions, gifts, entertainment or other unlawful expenses related to foreign or domestic political activity, (ii) made any unlawful payment to foreign or domestic government officials or employees or to any foreign or domestic political parties or campaigns from corporate funds, (iii) failed to disclose fully any contribution made by the Company (or made by any person acting on its behalf of which the Company is aware) which is in violation of law or (iv) violated in any material respect any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable non-U.S. anti-bribery Law.

4.19 Regulation M Compliance. The Company has not taken, directly or indirectly, any action designed to or that would reasonably be expected to cause or result in stabilization or manipulation of the price of the Common Stock to facilitate the sale or resale of the Shares.

4.20 Office of Foreign Assets Control. Neither the Company nor, to the Company's knowledge, any director, officer, agent, employee or Affiliate of the Company is currently subject to any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Treasury Department.

4.21 Development Matters.

(a) All preclinical and clinical studies conducted by or on behalf of the Company to support approval for commercialization of the Company's products or product candidates have been conducted by the Company, or to the Company's knowledge

by third parties, in compliance with all applicable federal, state or foreign laws, rules, orders and regulations, except for such failure or failures to be in compliance which would not reasonably be expected to have, singularly or in the aggregate, a Material Adverse Effect.

(b) The studies, tests and preclinical or clinical trials conducted by or on behalf of the Company that are described in the Company SEC Documents (the "**Company Studies and Trials**") were and, if still pending, are being, conducted in all material respects in accordance with experimental protocols, procedures and controls pursuant to, where applicable, accepted professional scientific standards; the descriptions of the results of the Company Studies and Trials contained in the Company SEC Documents are accurate in all material respects; the Company has no knowledge of any other studies or trials not described in the Company SEC Documents, the results of which are inconsistent with or call in question the results described or referred to in the Company SEC Documents; and, except as disclosed in the Company SEC Documents, the Company has not received any notices or correspondence from the United States Food and Drug Administration (the "**FDA**") or any foreign, state or local governmental authority exercising comparable authority requiring the termination, suspension or material modification of any Company Studies and Trials that termination, suspension or material modification would reasonably be expected to have a Material Adverse Effect and, to the Company's knowledge, there are no reasonable grounds for the same. The Company has obtained (or caused to be obtained) informed consent by or on behalf of each human subject who participated in the Company Studies and Trials. To the Company's knowledge, none of the Company Studies and Trials involved any investigator who has been disqualified as a clinical investigator or has been found by the FDA to have engaged in scientific misconduct. To the Company's knowledge, the manufacturing facilities and operations of its suppliers are operated in compliance in all material respects with all applicable statutes, rules, regulations and policies of the FDA and comparable governmental authorities outside of the United States to which the Company is subject.

4.22 Intellectual Property. The Company owns, possesses, or can acquire on reasonable terms the right to use all (i) patents, patent applications, trademarks, trademark registrations, service marks, service mark registrations, Internet domain name registrations, copyrights, copyright registrations, licenses and trade secret rights (collectively, "**Intellectual Property Rights**") and (ii) inventions, software, works of authorships, trademarks, service marks, trade names, databases, formulae, know how, Internet domain names and other intellectual property (including trade secrets and other unpatented and/or unpatentable proprietary confidential information, systems, or procedures) (collectively, "**Intellectual Property Assets**") necessary to conduct its business as currently conducted, and as proposed to be conducted and described in the Company SEC Documents. The Company has not received any opinion from its legal counsel concluding that any activities of its business infringes, misappropriates, or otherwise violates, valid and enforceable Intellectual Property Rights of any other person, and has not received written notice of any challenge, which is to its knowledge still pending, by any other person to the rights of the Company with respect to any Intellectual Property Rights or Intellectual Property Assets owned or used by the Company. To the

Company's knowledge, the Company's business as now conducted does not give rise to any infringement of, any misappropriation of, or other violation of, any valid and enforceable Intellectual Property Rights of any other person. All licenses for the use of the Intellectual Property Rights described in the Company SEC Documents are valid, binding upon, and enforceable by or against the Company, and to the Company's knowledge, by or against the parties thereto in accordance with their terms. The Company has complied in all material respects with, and is not in breach of, nor has it received any asserted or threatened claim of breach of any intellectual property licenses for the use of the Intellectual Property Rights, and the Company has no knowledge of any breach or anticipated breach by any other person of any such intellectual property licenses. Except as disclosed in the Company SEC Documents, no claim has been made or is pending against the Company alleging the infringement by the Company of any patent, trademark, service mark, trade name, copyright, trade secret, license in or other intellectual property right or franchise right of any person. The Company has taken reasonable steps to protect, maintain and safeguard its Intellectual Property Rights, including the execution of appropriate nondisclosure and confidentiality agreements. The consummation of the transactions contemplated by this Agreement will not result in the loss or impairment of or payment of any additional amounts with respect to, nor require the consent of any other person in respect of, the Company's right to own, use, or hold for use any of the Intellectual Property Rights as owned, used or held for use in the conduct of the business as currently conducted. The Company has at all times complied in all material respects with all applicable laws relating to privacy, data protection, and the collection and use of personal information collected, used, or held for use by the Company in the conduct of the Company's business. No claims have been asserted or threatened against the Company alleging a violation of any person's privacy or personal information or data rights and the consummation of the transactions contemplated hereby will not breach or otherwise cause any violation of any law related to privacy, data protection, or the collection and use of personal information collected, used, or held for use by the Company in the conduct of the Company's business. The Company takes reasonable measures to ensure that such information is protected against unauthorized access, use, modification or other misuse. The Company has taken all necessary actions to secure and record its ownership of all works of authorship and inventions made by its employees, consultants and contractors with an obligation of assignment during the time they were employed by or under contract with the Company and which relate to the Company's business. All founders and key employees have signed confidentiality and invention assignment agreements with the Company.

4.23 Real and Personal Property. The Company has good and marketable title in fee simple (in the case of real property) to, or has valid and marketable rights to lease or otherwise use, all items of real or personal property which are material to the business of the Company taken as a whole, in each case free and clear of all liens, encumbrances, security interests, claims and defects that do not, singularly or in the aggregate, materially affect the value of such property and do not interfere with the use made and proposed to be made of such property by the Company; and all of the leases and subleases material to the business of the Company, and under which the Company holds properties described in the Company SEC Documents, are in full force and effect and the

Company has not received any notice of any material claim of any sort that has been asserted by anyone adverse to the rights of the Company under any of the leases or subleases mentioned above, or affecting or questioning the rights of the Company to the continued possession of the leased or subleased premises under any such lease or sublease.

4.24 Labor and Employment. There is (a) no unfair labor practice complaint pending against the Company, nor to the Company's knowledge, threatened against it, before the National Labor Relations Board, any state or local labor relations board or any foreign labor relations board, and no significant grievance or significant arbitration proceeding arising out of or under any collective bargaining agreement is so pending against the Company, or, to the Company's knowledge, threatened against it and (b) no labor disturbance by or dispute with, employees of the Company exists or, to the Company's knowledge, is contemplated or threatened, and the Company is not aware of any existing or imminent labor disturbance by the employees of any of its principal suppliers, manufacturers, customers or contractors, that would reasonably be expected, singularly or in the aggregate, to have a Material Adverse Effect. The Company is not aware that any key employee or significant group of employees of the Company plans to terminate employment with the Company.

4.25 ERISA Matters. No "prohibited transaction" (as defined in Section 406 of the Employee Retirement Income Security Act of 1974, as amended, including the regulations and published interpretations thereunder ("**ERISA**"), or Section 4975 of the Internal Revenue Code of 1986, as amended from time to time (the "**Code**")) or "accumulated funding deficiency" (as defined in Section 302 of ERISA) or any of the events set forth in Section 4043(b) of ERISA (other than events with respect to which the thirty (30)-day notice requirement under Section 4043 of ERISA has been waived) has occurred or could reasonably be expected to occur with respect to any employee benefit plan of the Company which would, singularly or in the aggregate, reasonably be expected to have a Material Adverse Effect. Each employee benefit plan of the Company is in compliance in all material respects with applicable law, including ERISA and the Code. The Company has not incurred and would not reasonably be expected to incur liability under Title IV of ERISA with respect to the termination of, or withdrawal from, any pension plan (as defined in ERISA). Each pension plan for which the Company would have any liability that is intended to be qualified under Section 401(a) of the Code is so qualified, and nothing has occurred, whether by action or by failure to act, which would, singularly or in the aggregate, reasonably be expected to cause the loss of such qualification.

4.26 Environmental Matters. The Company is in compliance in all material respects with all foreign, federal, state and local rules, laws and regulations relating to the use, treatment, storage and disposal of hazardous or toxic substances or waste and protection of health and safety or the environment which are applicable to its businesses (the "**Environmental Laws**"). There has been no storage, generation, transportation, handling, treatment, disposal, discharge, emission, or other release of any kind of toxic or other wastes or other hazardous substances by, due to, or caused by the Company (or, to the Company's knowledge, any other entity for whose acts or omissions the Company is or may otherwise be liable) upon any of the property now or previously

owned or leased by the Company, or upon any other property, in violation of any law, statute, ordinance, rule, regulation, order, judgment, decree or permit or which would, under any law, statute, ordinance, rule (including rule of common law), regulation, order, judgment, decree or permit, give rise to any liability; and there has been no disposal, discharge, emission or other release of any kind on to such property or into the environment surrounding such property of any toxic or other wastes or other hazardous substances.

4.27 Taxes. The Company (i) has timely filed all necessary federal, state, local and foreign tax returns (or timely filed extensions with respect to such returns), and all such returns were true, complete and correct, (ii) has paid all federal, state, local and foreign taxes, assessments, governmental or other charges due and payable for which it is liable, including, without limitation, all sales and use taxes and all taxes which the Company is obligated to withhold from amounts owing to employees, creditors and third parties, and (iii) does not have any tax deficiency or claims outstanding or assessed or, to its knowledge, proposed against it, except those, in each of the cases described in clauses (i), (ii) and (iii) above, that would not, singularly or in the aggregate, reasonably be expected to have a Material Adverse Effect. The Company has not engaged in any transaction which is a corporate tax shelter or which could be characterized as such by the Internal Revenue Service or any other taxing authority. The accruals and reserves on the books and records of the Company in respect of tax liabilities for any taxable period not yet finally determined are adequate to meet any assessments and related liabilities for any such period, and since January 1, 2021, the Company has not incurred any liability for taxes other than in the ordinary course.

4.28 Insurance. The Company carries or is covered by insurance in such amounts and covering such risks as is adequate for the conduct of its business and the value of its properties and as is customary for companies engaged in similar businesses, at a similar stage of development, in similar industries. The Company has no reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage from similar insurers as may be necessary to continue its business at a cost that would not reasonably be expected to have a Material Adverse Effect. All policies of insurance owned by the Company are, to the Company's knowledge, in full force and effect and the Company is in compliance in all material respects with the terms of such policies. The Company has not received written notice from any insurer, agent of such insurer or the broker of the Company that any material capital improvements or any other material expenditures (other than premium payments) are required or necessary to be made in order to continue such insurance. Except for customary deductibles, the Company does not insure risk of loss through any captive insurance, risk retention group, reciprocal group or by means of any fund or pool of assets specifically set aside for contingent liabilities other than as described in the Company SEC Documents.

5. Representations and Warranties of the Investor. The Investor hereby represents and warrants to the Company that:

5.1 Organization; Good Standing. The Investor is a corporation duly organized, validly existing and in good standing under the laws of the State of Delaware. The Investor has all requisite corporate power and corporate authority to enter into the

Transaction Agreements, to purchase the Shares and to perform its obligations under and to carry out the other transactions contemplated by the Transaction Agreements.

5.2 Authorization.

(a) The Investor has full right, power and authority to execute and deliver the Transaction Agreements and the Collaboration Agreement and to perform its obligations hereunder and thereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of each of the Transaction Agreements and the Collaboration Agreement and the consummation by it of the transactions contemplated thereby has been duly and validly taken.

(b) The Transaction Agreements and the Collaboration Agreement have been duly executed and delivered by the Investor and, upon the due execution and delivery of the Transaction Agreements and the Collaboration Agreement by the Company, will constitute valid and legally binding obligations of the Investor, enforceable against the Investor in accordance with their respective terms, except with respect to the Enforceability Exceptions.

5.3 No Conflicts. The execution, delivery and performance of the Transaction Agreements and the Collaboration Agreement, the subscription for and purchase of the Shares and the consummation of the transactions contemplated by the Transaction Agreements and the Collaboration Agreement will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, or result in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Investor pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Investor is a party, by which the Investor is bound or to which any of the property or assets of the Investor is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Investor or (iii) result in the violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority having jurisdiction over the Investor or any of its subsidiaries, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation or default that would not, individually or in the aggregate, have a material adverse effect on the Investor's ability to perform its obligations or consummate the Transaction in accordance with the terms of this Agreement.

5.4 No Governmental Authority or Third-Party Consents. No consent, approval, authorization, order, license, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Investor of each of the Transaction Agreements or the Collaboration Agreement or with the subscription for and purchase of the Shares, except as required pursuant to the HSR Act.

5.5 Purchase Entirely for Own Account. The Investor acknowledges that the Shares shall be acquired for investment for the Investor's own account, not as a nominee or agent, and not with a view to the resale or distribution of any part thereof, and

the Investor has no present intention of selling, granting any participation or otherwise distributing the Shares. The Investor can bear the economic risk of an investment in the Shares indefinitely and a total loss with respect to such investment. The Investor does not have and will not have as of the Closing any contract, undertaking, agreement, arrangement or understanding with any Person to sell, transfer or grant participation to a Person any of the Shares.

5.6 Disclosure of Information. The Investor has received or has had full access to all the information from the Company and its management that the Investor considers necessary or appropriate for deciding whether to purchase the Shares hereunder. The Investor further represents that it has had an opportunity to ask questions and receive answers from the Company regarding the Company, its financial condition, results of operations and prospects and the terms and conditions of the offering of the Shares sufficient to enable it to evaluate its investment.

5.7 Investment Experience and Accredited Investor Status. The Investor is an "accredited investor" (as defined in Regulation D under the Securities Act). The Investor has such knowledge and experience in financial or business matters that it is capable of evaluating the merits and risks of the investment in the Shares to be purchased hereunder.

5.8 Acquiring Person. As of the Signing Date, the Investor beneficially owns (as determined pursuant to Rule 13d-3 under the Exchange Act without regard for the number of days in which a Person has the right to acquire such beneficial ownership, and without regard to Investor's rights under this Agreement) 4,179,728 shares of the Common Stock (the "**2019 Shares**"). Other than the 2019 Shares, as of the Signing Date, neither the Investor nor any of its Affiliates beneficially owns, and immediately prior to the Closing, neither the Investor nor any of its Affiliates will beneficially own (in each case, as determined pursuant to Rule 13d-3 under the Exchange Act without regard for the number of days in which a Person has the right to acquire such beneficial ownership, and without regard to Investor's rights under this Agreement), any securities of the Company, except for securities that may be beneficially owned by employee benefit plans of either the Investor or any of its Affiliates. All securities owned by the Investor or any of its Affiliates that are required to be reported in accordance with the reporting requirements of Section 16 of the Exchange Act and the rules and regulations promulgated thereunder have been duly reported in such filings.

5.9 No "Bad Actor" Disqualification. The Investor has not taken any of the actions set forth in, and is not subject to, the disqualification provisions of Rule 506(d)(1) of the Securities Act. The Investor's responses in the questionnaire delivered to the Company by the Investor related to qualification under Rule 506(d)(1) are true and correct as of the Signing Date and will remain true and correct as of the Closing Date.

5.10 Restricted Securities. The Investor understands that the Shares, when issued, shall be "restricted securities" under the federal securities Laws inasmuch as they are being acquired from the Company in a transaction not involving a public offering and that under such Laws the Shares may be resold without registration under the Securities

Act only in certain limited circumstances. The Investor represents that it is familiar with Rule 144, as presently in effect. The Investor understands that the Shares are being offered and sold to it in reliance on specific exemptions from the registration requirements of United States federal and state securities Laws and the Company is relying in part upon the truth and accuracy of, and the Investor's compliance with, the representations, warranties, agreements, acknowledgements and understandings of the Investor set forth in this Agreement in order to determine the availability of such exemptions and the eligibility of the Investor to acquire the Shares.

5.11 Legends. The Investor understands that any certificates or ledger entries representing the Shares shall bear the following legends:

(a) "THESE SECURITIES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF A REGISTRATION STATEMENT IN EFFECT WITH RESPECT TO THE SECURITIES UNDER THE SECURITIES ACT OR AN OPINION OF COUNSEL (WHICH COUNSEL SHALL BE REASONABLY SATISFACTORY TO THE COMPANY) THAT SUCH REGISTRATION IS NOT REQUIRED OR UNLESS SOLD PURSUANT TO RULE 144 OF THE SECURITIES ACT.";

(b) "THESE SECURITIES ARE SUBJECT TO AND SHALL BE TRANSFERABLE ONLY UPON THE TERMS AND CONDITIONS OF AN AMENDED AND RESTATED INVESTOR AGREEMENT DATED AS OF JANUARY 8, 2023, BY AND BETWEEN VOYAGER THERAPEUTICS, INC. AND NEUROCRINE BIOSCIENCES, INC., A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF VOYAGER THERAPEUTICS, INC."; and

(c) any legend required by applicable state securities Laws or the other Transaction Agreements.

5.12 Financial Assurances. As of the Signing Date, the Investor has, and as of the Closing Date, the Investor will have, access to cash in an amount sufficient to pay to the Company the Aggregate Purchase Price.

5.13 SEC Reports. The Investor has reviewed the Company SEC Documents.

6. Investor's Conditions to Closing. The Investor's obligation to purchase the Shares at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by the Investor):

6.1 Representations and Warranties. The representations and warranties made by the Company in Section 4 hereof shall be true and correct as of the Signing Date and as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date;

provided, however, that for purposes of this Section 6.1, all such representations and warranties of the Company (other than Sections 4.1, 4.2, 4.3, 4.4, 4.5, 4.6, 4.8, and 4.11 hereof) shall be deemed to be true and correct for purposes of this Section 6.1 unless the failure or failures of such representations and warranties to be so true and correct, without regard to any "material," "materiality" or "Material Adverse Effect" qualifiers set forth therein, constitute a Material Adverse Effect.

6.2 Representations and Warranties in the Collaboration Agreement. The representations and warranties made by the Company in Section 12.2 of the Collaboration Agreement shall be true and correct as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date; provided, however, that for purposes of this Section 6.2, all such representations and warranties of the Company shall be deemed to be true and correct for purposes of this Section 6.2 unless the failure or failures of such representations and warranties to be so true and correct, without regard to any "material" or "materiality" qualifiers set forth therein, individually or in the aggregate, has had or would reasonably be expected to have a Material Adverse Effect.

6.3 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by the Company on or prior to the Closing Date shall have been performed or complied with in all material respects.

6.4 Investor Agreement. The Investor Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

6.5 Collaboration Agreement. The Collaboration Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect as of the Closing Date.

6.6 No Material Adverse Effect. From and after the Signing Date until the Closing Date, there shall have occurred no event that has caused a Material Adverse Effect.

6.7 Listing. The Shares shall be eligible and approved for listing on the Nasdaq Stock Market.

7. Company's Conditions to Closing. The Company's obligation to issue and sell the Shares at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by the Company):

7.1 Representations and Warranties. The representations and warranties made by the Investor in Section 5 hereof shall be true and correct as of the Signing Date and as of the Closing Date as though made on and as of such Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date.

7.2 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by the Investor on or prior to the Closing Date shall have been performed or complied with in all material respects.

7.3 Investor Agreement. The Investor Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

7.4 Collaboration Agreement. The Collaboration Agreement shall not have been terminated in accordance with its terms and shall be in full force and effect.

8. Mutual Conditions to Closing. The obligations of the Investor and the Company to consummate the Closing are subject to the fulfillment as of the Closing Date of the following conditions:

8.1 HSR Act Qualification. Any required HSR Clearances shall have been obtained.

8.2 Absence of Litigation. There shall be no action, suit, proceeding or investigation by a Governmental Authority pending or currently threatened in writing against the Company or the Investor (i) that questions (A) the validity of any Transaction Agreement or (B) the right of the Company or the Investor to enter into any Transaction Agreement or to consummate the transactions contemplated hereby or thereby or (ii) which, if determined adversely, would impose substantial monetary damages on the Company or the Investor as a result of the consummation of the transactions contemplated by any Transaction Agreement.

8.3 No Prohibition. No provision of any applicable Law and no judgment, injunction (preliminary or permanent), order or decree that prohibits, makes illegal or enjoins the consummation of the Transaction shall be in effect.

9. Termination.

9.1 Ability to Terminate. This Agreement may be terminated at any time prior to the Closing by:

(a) mutual written consent of the Company and the Investor;

(b) either the Company or the Investor, upon written notice to the other, if any of the mutual conditions to the Closing set forth in Section 8 hereof shall have become incapable of fulfillment by the Termination Date and shall not have been waived in writing by the other party within ten business days after receiving receipt of written notice of an intention to terminate pursuant to this clause (b); provided, however, that the right to terminate this Agreement under this Section 9.1(b) shall not be available to any party whose failure to fulfill any obligation under this Agreement has been the cause of, or resulted in, the failure to consummate the transactions contemplated hereby prior to the Termination Date;

(c) the Company, upon written notice to the Investor, so long as the Company is not then in breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 6.1, 6.2, 6.3, 6.4 or 6.5 hereof, as applicable, could not be satisfied by the Termination Date, (i) upon a material breach of any covenant or agreement on the part of the Investor set forth in this Agreement, or (ii) if any representation or warranty of the Investor shall have been or become untrue, in each case such that any of the conditions set forth in Section 7.1, 7.2, 7.3 or 7.4 hereof, as applicable, could not be satisfied by the Termination Date;

(d) the Investor, upon written notice to the Company, so long as the Investor is not then in breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 7.1, 7.2, 7.3, or 7.4 hereof, as applicable, could not be satisfied by the Termination Date, (i) upon a material breach of any covenant or agreement on the part of the Company set forth in this Agreement, or (ii) if any representation or warranty of the Company shall have been or become untrue, in each case such that any of the conditions set forth in Section 6.1, 6.2, 6.3, 6.4 or 6.5 hereof, as applicable, could not be satisfied by the Termination Date.

9.2 Effect of Termination. In the event of the termination of this Agreement pursuant to Section 9.1 hereof, (i) this Agreement (except for this Section 9.2 and Section 11 hereof (other than Section 11.12), and any definitions set forth in this Agreement and used in such sections) shall forthwith become void and have no effect, without any liability on the part of any party hereto or its Affiliates, and (ii) all filings, applications and other submissions made pursuant to this Agreement, to the extent practicable, shall be withdrawn from the agency or other Person to which they were made or appropriately amended to reflect the termination of the transactions contemplated hereby; provided, however, that nothing contained in this Section 9.2 shall relieve any party from liability for fraud or any intentional or willful breach of this Agreement.

10. Additional Covenants and Agreements.

10.1 Market Listing. From the Signing Date through the Closing Date, Company shall use all commercially reasonable efforts to (i) maintain the listing and trading of the Common Stock on the Nasdaq Stock Market and (ii) effect the listing of the Shares on the Nasdaq Stock Market, including submitting the LAS to the Nasdaq Stock Market no later than fifteen (15) calendar days prior to the Closing Date.

10.2 Notification under the HSR Act. Each party will use reasonable efforts to do, or cause to be done, all things necessary, proper and advisable to, as promptly as practicable, take all actions necessary to obtain expiration or termination of all applicable waiting periods and requests for information (and any extensions thereof) under the HSR Act, including filing with the FTC and Antitrust Division of the DOJ, any HSR Filing required of it under the HSR Act with respect to the transactions contemplated hereby within thirty (30) days after the Signing Date (or such later time as may be agreed to in writing by the parties). The parties shall cooperate with one another to the extent necessary in the preparation of any such HSR Filing and during the review by the FTC or the Antitrust Division of the DOJ. Each party shall be responsible for its own costs and

expenses; provided, however, that the Investor shall be solely responsible for any fees (other than penalties that may be incurred as a result of actions or omissions on the part of the Company) required to be paid to any Governmental Agency in connection with making any such HSR Filing. If the parties make an HSR filing hereunder, then this Agreement shall terminate at the election of either party, immediately upon notice to the other party, if the FTC or the DOJ seeks a preliminary injunction (or its equivalent) to enjoin the transactions contemplated hereby and thereby or the FTC issues a complaint pursuant to Section 5(b) of the FTC Act in connection therewith. In the event of such termination, this Agreement shall be of no further force and effect.

10.3 Assistance and Cooperation. Prior to the Closing, upon the terms and subject to the conditions set forth in this Agreement, each of the parties agrees to use all reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, and to assist and cooperate with the other party in doing, all things necessary, proper or advisable to consummate and make effective, in the most expeditious manner practicable, the transactions contemplated by this Agreement, including using all reasonable efforts to accomplish the following: (i) taking all reasonable acts necessary to cause the conditions precedent set forth in Sections 6, 7 and 8 hereof to be satisfied (including, in the case of the Company, promptly notifying the Investor of any notice from the Nasdaq Stock Market with respect to the LAS); (ii) taking all reasonable actions necessary to obtain all necessary actions or non-actions, waivers, consents, approvals, orders and authorizations from Governmental Authorities and the making of all necessary registrations, declarations and filings (including registrations, declarations and filings with Governmental Authorities, if any); (iii) taking all reasonable actions necessary to obtain all necessary consents, approvals or waivers from Third Parties; and (iv) except as otherwise provided for in Section 10.2 hereof, defending any suits, claims, actions, investigations or proceedings, whether judicial or administrative, challenging this Agreement or the consummation of the transactions contemplated hereby, including seeking to have any stay or temporary restraining order entered by any court or other Governmental Authority vacated or reversed.

10.4 Legend Removal.

(a) Certificates or ledger entries evidencing the Shares shall not contain the legend set forth in Section 5.11(a) hereof: (i) following a sale of such Shares pursuant to a registration statement covering the resale of such Shares, while such registration statement is effective under the Securities Act, (ii) following any sale of such Shares pursuant to Rule 144, (iii) if such Shares are eligible for sale under Rule 144, without the requirement for the Company to be in compliance with the current public information required under Rule 144 as to such Shares and without volume or manner-of-sale restrictions under Rule 144 or (iv) if such legend is not required under applicable requirements of the Securities Act (including judicial interpretations and pronouncements issued by the staff of the SEC).

(b) Certificates or ledger entries evidencing the Shares shall not contain the legend set forth in Section 5.11(b) hereof following: (i) a sale of such Shares pursuant to a registration statement covering the resale of such Shares, while such

registration statement is effective under the Securities Act, (ii) any sale of such Shares pursuant to Rule 144 or (iii) the expiration of the Standstill Term (as defined in the Investor Agreement), the Lock-Up Term (as defined in the Investor Agreement) and the Voting Agreement Term (as defined in the Investor Agreement); provided that any transfer described in clause (i) or (ii) above shall have been in compliance with all applicable provisions of the Investor Agreement.

(c) The Company agrees that at such time as any legend set forth in Section 5.11 hereof is no longer required under this Section 10.4, the Company will, no later than three (3) Business Days following the delivery by the Investor to the Company or notice by the Investor to the Company of either the delivery by the Investor to the Transfer Agent of a certificate representing Shares issued with such legend or, in the event such shares are uncertificated, notice of the Investor's desire to remove such legend(s) that are no longer required (together with any legal opinion required by the Transfer Agent), deliver or cause to be delivered to the Investor a certificate representing such Shares that is free from such legend, or, in the event that such shares are uncertificated, remove or cause to be removed any such legend in the Company's stock records. The Company may not make any notation on its records or give instructions to the Transfer Agent that enlarge the restrictions on transfer set forth in Section 5.11 hereof.

10.5 Conduct of Business. During the period from the Signing Date until the Closing, except as consented to in writing by the Investor, the Company shall not (i) declare, set aside or pay any dividend or make any other distribution or payment (whether in cash, stock or property or any combination thereof) in respect of its capital stock, or establish a record date for any of the foregoing, or (ii) make any other actual, constructive or deemed distribution in respect of any shares of its capital stock or otherwise make any payments to stockholders in their capacity as such, except pursuant to repurchases of equity pursuant to the terms of its equity compensation plans.

11. Miscellaneous.

11.1 Governing Law; Submission to Jurisdiction. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Any action brought, arising out of, or relating to this Agreement shall be brought in the Court of Chancery of the State of Delaware. Each party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The parties hereby consent to and grant the Court of Chancery of the State of Delaware jurisdiction over such parties and over the subject matter of any such claim and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 11.3 hereof or in such other manner as may be permitted by law, shall be valid and sufficient thereof.

11.2 Waiver. Neither party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either party to assert a right hereunder or to insist upon compliance with any term of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

11.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit A attached hereto and shall be (i) delivered personally; (ii) sent by certified mail (return receipt requested), postage prepaid; or (iii) sent via a reputable nationwide overnight express courier service (signature required). Any such notice, instruction or communication shall be deemed to have been delivered (A) upon receipt if delivered by hand; (B) three (3) Business Days after it is sent by certified mail, return receipt requested, postage prepaid; or (C) one (1) Business Day after it is sent via a reputable nationwide overnight courier service. Either party may change its address by giving notice to the other party in the manner provided above; provided that notices of a change of address shall be effective only upon receipt thereof.

11.4 Entire Agreement. This Agreement, the Investor Agreement and the Collaboration Agreement, in each case together with the schedules and exhibits thereto, set forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the parties and supersede and terminate all prior agreements and understanding between the parties. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the parties other than as set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the parties unless reduced to writing and signed by the respective authorized officers of the parties.

11.5 Headings; Nouns and Pronouns; Section References. Headings and any table of contents used in this Agreement are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

11.6 Severability. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either party and to match

the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

11.7 Assignment. Except for an assignment of this Agreement or any rights hereunder by the Investor to an Affiliate, neither this Agreement nor any of the rights or obligations hereunder may be assigned by either the Investor or the Company without (i) the prior written consent of Company in the case of any assignment by the Investor or (ii) the prior written consent of the Investor in the case of an assignment by the Company.

11.8 Parties in Interest. All of the terms and provisions of this Agreement shall be binding upon, and shall inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

11.9 Counterparts. This Agreement may be signed in counterparts, each and every one of which shall be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

11.10 Third-Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto. No Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

11.11 No Strict Construction. This Agreement has been prepared jointly and will not be construed against either party.

11.12 Survival of Warranties. The representations and warranties of the Company and the Investor contained in this Agreement shall survive the Closing and the delivery of the Shares.

11.13 Remedies. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

11.14 Expenses. Each party shall pay its own fees and expenses in connection with the preparation, negotiation, execution and delivery of the Transaction Agreements.

11.15 No Publicity. The parties hereto agree that the provisions of Section 11.3 of the Collaboration Agreement shall be applicable to the parties to this Agreement with respect to any public disclosures regarding the proposed transactions contemplated by the Transaction Agreements and the Collaboration Agreement or regarding the parties hereto or their Affiliates (it being understood that the provisions of

Section 11.3 of the Collaboration Agreement shall be read to apply to disclosures of information relating to this Agreement and the transactions contemplated hereby).

(Signature Page Follows)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

NEUROCRINE BIOSCIENCES, INC.

By: /s/ Kevin Gorman
Name: Kevin Gorman
Title: CEO

VOYAGER THERAPEUTICS, INC.

By: /s/ Alfred W. Sandrock, Jr.
Name: Alfred W. Sandrock, Jr., M.D., Ph.D.
Title: President & CEO

(Signature Page to Stock Purchase Agreement)

SCHEDULE 1

LIST OF SUBSIDIARIES

1. Voyager Securities Corporation, a Massachusetts corporation

EXHIBIT A

NOTICES

If to the Investor:

Neurocrine Biosciences, Inc.
12780 El Camino Real
San Diego, CA 92130
Attention: General Counsel

with a copy to:

Cooley LLP
55 Hudson Yards
New York, NY 10001
Attention: Jason L. Kent, Esq.

If to the Company:

Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: Chief Executive Officer

with copies to:

Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: General Counsel

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, NY 10007
Attention: Brian A. Johnson, Esq.

Exhibit 10.45

EXECUTION COPY
CONFIDENTIAL

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

COLLABORATION AND LICENSE

AGREEMENT

by and between

VOYAGER THERAPEUTICS, INC.

AND

NEUROCRINE BIOSCIENCES, INC.

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EXHIBITS

Exhibit A Stock Purchase Agreement

COLLABORATION AND LICENSE AGREEMENT

This COLLABORATION AND LICENSE AGREEMENT (the "Agreement") is entered into as of January 8, 2023 (the "Execution Date"), by and between Voyager Therapeutics, Inc., a Delaware corporation having its principal place of business at 64 Sidney Street, Cambridge, MA 02139 ("Voyager"), and Neurocrine Biosciences, Inc., a Delaware corporation having its principal place of business at 12780 El Camino Real, San Diego, CA 92130 ("Neurocrine"). Voyager and Neurocrine are sometimes referred to herein individually as a "Party" and collectively as the "Parties".

RECITALS

WHEREAS, Voyager is a biotechnology company dedicated to breaking through barriers in gene therapy and neurology and possesses expertise in the research, development, manufacturing and commercialization of human therapeutics;

WHEREAS, Neurocrine is a biopharmaceutical company focused on developing and commercializing treatments for neurological and endocrine-related disorders, and possesses expertise in the research, development, manufacturing and commercialization of human therapeutics; and

WHEREAS, Voyager and Neurocrine desire to engage in a collaborative effort in which Voyager will carry out certain preclinical research activities and clinical development activities relating to the identification and development of gene therapy products directed to GBA1 (as defined below) and certain other genetic targets, and pursuant to which Neurocrine will have certain rights to further develop and commercialize such products.

NOW, THEREFORE, in consideration of the premises and mutual covenants herein contained, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

As used in this Agreement, the following terms will have the meanings set forth in this ARTICLE 1 unless context dictates otherwise:

1.1 "2019 CLA" has the meaning set forth in Section 11.1.

1.2 "AAA" has the meaning set forth in Section 15.3.2.

1.3 "AAV" means an adeno-associated virus, including its recombinant forms.

1.4 "Acquired Affiliate" has the meaning set forth in Section 9.3.1.

1.5 "Acquired Competing Product" has the meaning set forth in Section 9.3.1.

1.6 "Acquired Competing Program" has the meaning set forth in Section 9.3.1.

1.7 "Acquirer" has the meaning set forth in Section 1.28.

1.8 "Acquiring Entities" means any Person that becomes an Affiliate of a Party pursuant to a Change of Control effected after the Execution Date, and the Affiliates of such Party; but excluding the applicable Party and its Affiliates existing immediately prior to such Change of Control.

1.9 "Affiliate" means any Person that, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with a Party to this Agreement, regardless of whether such Affiliate is or becomes an Affiliate on or after the Execution Date, but only for so long as such control exists. A Person shall be deemed to "control" another Person if it: (a) owns, directly or indirectly, beneficially or legally, more than fifty percent (50%) of the outstanding voting securities or capital stock of such other Person, or has other comparable ownership interest with respect to any Person other than a corporation; or (b) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

1.10 "Agreement" has the meaning set forth in Preamble.

1.11 "Alliance Manager" has the meaning set forth in Section 3.7.

1.12 "Annual Net Sales" means, on a Product-by-Product basis, the total Net Sales of such Product in the U.S. or in the Territory outside the U.S., as applicable, in a particular Calendar Year.

1.13 "Antitrust Laws" means any law relating to competition that is enforced by the U.S. Federal Trade Commission or the Antitrust Division of the U.S. Department of Justice.

1.14 "Arising Capsid IP" means: (a) Arising IP Created jointly by Representatives of Neurocrine and Representatives of Voyager that constitutes Capsid IP; and (b) Arising IP Created solely by Representatives of Neurocrine through the use of Voyager's Confidential Information, including unpublished sequence information for the Voyager Capsid.

1.15 "Arising IP" means: (a) all Know-How Created by either or both Parties in the performance of the Discovery Activities or in the course of Development, Manufacture and Commercialization of Collaboration Candidates or Products; and (b) all Patent Rights Covering such Know-How.

1.16 "Assumption Notice" has the meaning set forth in Section 2.1.4.

1.17 "[**]" has the meaning set forth in Section 7.3.

1.18 "Biosimilar Product" means, with respect to a particular Product in a particular country in the Territory, any Gene Therapy Product sold by a Third-Party not authorized by or on behalf of Neurocrine, its Affiliates, or Sublicensees, that targets the same Target as the Product and, on the basis of a prior Regulatory Approval granted to a Product: (a) is approved by the FDA pursuant to Section 351(k) of the PHSA or successor thereto; (b) is approved by the EMA pursuant to EU Directive 2001/83/EC or successor thereto in the European Union or any member state

thereof citing such Product as the reference product; or (c) has received abbreviated Regulatory Approval from the applicable Regulatory Authority in another foreign jurisdiction.

1.19 "BLA" means a Biologics License Application submitted to the FDA pursuant to 21 U.S.C. §601.2 (or successor regulation thereto), for purposes of obtaining Regulatory Approval for a new biologic in the United States. References to BLA in this Agreement shall include any comparable filing(s) outside the U.S. for the purpose of obtaining Regulatory Approval in any other country or group of countries.

1.20 "Business Day" means a day on which banking institutions in Boston, Massachusetts or San Diego, California are open for business, excluding any Saturday or Sunday.

1.21 "Calendar Quarter" means a period of three (3) consecutive months ending on the last day of March, June, September, or December, respectively; provided that, the first Calendar Quarter starts on the Effective Date and ends on March 31, 2023.

1.22 "Calendar Year" means a period of twelve (12) consecutive months beginning on January 1 and ending on December 31; provided that the first Calendar Year starts on the Effective Date and ends on December 31, 2023.

1.23 "Capsid" means the protein shell of an AAV, consisting of oligomeric structural subunits made of certain proteins.

1.24 "Capsid IP" means all Capsid Know-How and Capsid Patent Rights.

1.25 "Capsid Know-How" means all Know-How that is related to any Voyager Capsid or any method of Manufacture or use of any Voyager Capsid; in each case, whether alone or in combination with any payload, including a Program Payload. Capsid Know-How shall be considered Voyager's Confidential Information except to the extent such Capsid Know-How relates to (a) any component of a Collaboration Candidate other than the Voyager Capsid therein; (b) any Program Target or Program Payload; or (c) any method of Manufacture or use of a Collaboration Candidate (and not only the Voyager Capsid therein) or Program Payload.

1.26 "Capsid Patent Rights" means any Patent Rights that Cover any Voyager Capsid or any other Capsid Know-How.

1.27 "cGMP" means the current Good Manufacturing Practices as provided for (and as amended from time to time) in the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) Harmonized Tripartite Guideline, Good Manufacturing Practice Guide for Active Pharmaceutical Ingredients, Q7 (ICH Q7), and the United States Code of Federal Regulations 21 CFR Parts 210 and 211, or any similar regulation in other applicable jurisdictions.

1.28 "Change of Control" means, with respect to a Party: (a) the acquisition of beneficial ownership, directly or indirectly, by any Third-Party of securities or other voting interest of such Party representing a majority or more of the combined voting power of such Party's then outstanding securities or other voting interests; (b) any merger, consolidation or business

combination involving such Party with a Third-Party that results in the holders of beneficial ownership (other than by virtue of obtaining irrevocable proxies) of the voting securities or other voting interests of such Party (or, if applicable, the ultimate parent of such Party) immediately prior to such merger, consolidation or business combination ceasing to hold beneficial ownership of more than fifty percent (50%) of the combined voting power of the surviving entity immediately after such merger, consolidation or business combination; or (c) any sale, lease, exchange, contribution or other transfer to a Third-Party (in one transaction or a series of related transactions) of all or substantially all of the assets of such Party to which this Agreement relates. The acquiring or combining Third-Party in any of clause (a), (b) or (c), is referred to herein as the "Acquirer".

1.29 "Clinical Trial" means a Phase 1 Clinical Trial, Phase 2 Clinical Trial, Phase 3 Clinical Trial or any other study in which human subjects or patients are dosed with a drug, whether approved or investigational.

1.30 "CNS Target" means a Target whose modulation is reasonably believed to ameliorate a disease of the central nervous system.

1.31 "Co-Co Agreement" has the meaning set forth in Section 4.1.1.

1.32 "Co-Co Option" has the meaning set forth in Section 4.1.1.

1.33 "Co-Co Product" has the meaning set forth in Section 4.1.1.

1.34 "Co-Co Program" has the meaning set forth in Section 4.1.1.

1.35 "Co-Co Territory" has the meaning set forth in Section 4.1.1.

1.36 "Co-Co Trigger Date" has the meaning set forth in Section 4.1.1.

1.37 "Collaboration" has the meaning set forth in Section 2.1.1.

1.38 "Collaboration Candidate" means any Gene Therapy Product that: (a) includes a Voyager Capsid and Program Payload; and (b) is Developed under a Program.

1.39 "Collaboration IP Working Group" has the meaning set forth in Section 3.3.1(a).

1.40 "Combination Product" has the meaning set forth in Section 1.116.

1.41 "Commercialization" and "Commercialize" means any and all activities undertaken relating to the marketing, obtaining pricing and reimbursement approvals, promotion (including advertising, detailing or continuing medical education, including medical education with respect to disease states, and including prior to Regulatory Approval of the applicable product), any other offering for sale or any sale of a product, including any distribution, importation, exportation or transport of a product for sales purposes. "Commercialization" shall not include Development or Manufacturing.

1.42 "Commercial Milestones" means the Milestone Events described in Section 8.2.3.

1.43 "Commercially Reasonable Efforts" means, with respect to the efforts to be expended by a Party with respect to an agreed objective, such reasonable, diligent, and good faith efforts that a biopharmaceutical company of similar size would normally use taking into account the reasonable allocation of such company's resources under the circumstances to accomplish a similar objective for its own internally developed product that is of similar market potential at a similar stage in its Development, Commercialization or product life, taking into account all relevant factors, including: (a) the potential profitability of the product; (b) the costs and risks of Developing, Manufacturing, having Manufactured, using and Commercializing the product; (c) scientific, safety and regulatory concerns; (d) product profile; (e) the competitiveness of the marketplace; and (f) the proprietary position of the product. In addition, "Commercially Reasonable Efforts" shall be determined on a country-by-country or market-by-market basis (as most applicable) for a particular product, and it is anticipated that the level of effort will change over time, including to reflect changes in the status of the product and the countries (or markets) involved. Where a Party has an obligation to use Commercially Reasonable Efforts, the efforts of such Party and its Affiliates, subcontractors and Sublicensees shall be considered in determining whether such Party has satisfied such obligation.

1.44 "Committee" has the meaning set forth in Section 3.3.1.

1.45 "Common Stock" means shares of Voyager common stock, par value $0.001 per share.

1.46 "Competitive Infringement" has the meaning set forth in Section 10.3.1.

1.47 "Competitive Product" means a Gene Therapy Product (other than a Product) that is directed to the GBA1 Target or any New Discovery Target; provided, however, Competitive Product specifically excludes a Gene Therapy Product that is [**].

1.48 "Confidential Information" has the meaning set forth in Section 11.1.

1.49 "Control" means, subject to Section 5.2.3, with respect to a Person and any Know-How or Patent Right, the possession by such Person of the right (whether through ownership or license (other than by a license under this Agreement) to grant the licenses or sublicenses as provided herein without violating the terms of any agreement or other arrangement with any Third-Party. Notwithstanding anything in this Agreement to the contrary, any Patent Rights or Know-How controlled by any Acquiring Entity of a Party will not be deemed to be "Controlled" by such Party for purposes of this Agreement, unless such Patent Rights or Know-How (a) were developed, invented or obtained with the use of any non-public Know-How in the Voyager IP (if such Party is Voyager) or Neurocrine IP (if such Party is Neurocrine) or (b) are used to conduct any Discovery Activities or activities under the Co-Co Agreement.

1.50 "Cover" means, means with regard to a particular subject matter and a Patent Right, that, in the absence of ownership of or a license granted under such Patent Right, the making, having made, use, offer for sale, sale, importation, Development, Manufacture, or Commercialization of such subject matter, would infringe (or, with respect to a claim in a pending patent application, would infringe if such claim were to issue) a claim of such Patent Right.

1.51 "CPI" has the meaning set forth in Section 1.80.

1.52 "Created" means: (a) with respect to any Know-How constituting an Invention, invented in accordance with U.S. patent laws; or (b) with respect to any other Know-How, authored, discovered, developed or created.

1.53 "Defense Proceeding" has the meaning set forth in Section 10.2.2(a)(i).

1.54 "Delivery Event" has the meaning set forth in Section 5.7.

1.55 "Develop" or "Development" means non-clinical, pre-clinical and clinical research and development activities, including discovery, identification, research, engineering, characterization, development, modification, optimization, drug metabolism and pharmacokinetics, translational research, toxicology, pharmacology toxicology studies, statistical analysis and report writing, formulation development and optimization, Clinical Trials, regulatory affairs (including preparation for a Regulatory Approval Application submission and other submission-related activities), product approval and registration activities, and all other activities necessary to conduct IND-enabling studies, conduct Clinical Trials, or seek, obtain and maintain Regulatory Approval. "Development" shall not include Commercialization but may include certain activities relating to the development of Manufacturing process (such as formulation, process development, Manufacturing scale-up, and related regulatory activities) to the extent applicable.

1.56 "Development Candidate" means on a Program-by-Program basis, a Collaboration Candidate either: (a) that has been determined by the JSC to meet the Development Candidate Criteria pursuant to Section 2.1.6; (b) that has otherwise been selected by the JSC as a Development Candidate pursuant to Section 2.1.6; or (c) for which Neurocrine or an Affiliate or Sublicensee has initiated an IND-enabling GLP toxicity study with such Collaboration Candidate, outside of the process under Section 2.1.6.

1.57 "Development Candidate Criteria" means: (a) with respect to the GBA1 Program, the criteria developed by the JSC after the Effective Date pursuant to Section 3.1.2(e); and (b) with respect to each New Discovery Program, the criteria developed by the JSC and set forth in the applicable New Discovery Program Development Plan.

1.58 "Development Costs" means the FTE Costs (at the then-current FTE Rate) and the Out-of-Pocket Costs (without markup) incurred by or on behalf of a Party or any of its Affiliates in the conduct of the Development of Collaboration Candidates or Products.

1.59 "Development Milestones" means the Milestone Events described in Sections 8.2.1 and 8.2.2.

1.60 "Development Plan" means: (a) the GBA1 Development Plan; or (b) any New Discovery Program Development Plan; as applicable.

1.61 "Disclosing Party" has the meaning set forth in Section 11.1.

1.62 "Discovery Activities" means the following activities, as undertaken pursuant to a Development Plan during the Discovery Period: (a) the discovery of Voyager Capsids and Collaboration Candidates; and (b) any other non-clinical activities relating to Development, Manufacture or Commercialization of Collaboration Candidates and Products as set forth in the applicable Development Plan.

1.63 "Discovery Period" means the period beginning on the Effective Date and ending on the third (3rd) anniversary of the Effective Date, which may be extended upon mutual written agreement of the Parties.

1.64 "Dispute" has the meaning set forth in Section 15.2.

1.65 "Dollars" or "$" means the legal tender of the U.S.

1.66 "Effective Date" means the HSR Clearance Date.

1.67 "EMA" means the European Medicines Agency, and any successor entity thereto.

1.68 "Exclusive Capsid" has the meaning set forth in Section 2.1.8(b).

1.69 "Exclusivity-Eligible Capsid" has the meaning set forth in Section 2.1.8(a).

1.70 "Execution Date" has the meaning set forth in Preamble.

1.71 "Executive Officers" means the Chief Executive Officer, in the case of Voyager, and the Chief Executive Officer, in the case of Neurocrine, or in each case, any designee of such person that is approved by the other Party in writing.

1.72 "Existing Confidentiality Agreement" has the meaning set forth in Section 11.1.

1.73 "Existing In-License Agreement" means each of the in-licenses of Voyager or any of its Affiliates listed in Schedule 1.73.

1.74 "Exploit" or "Exploitation" means to make, have made, import, use, sell, or offer for sale, Develop, Manufacture or Commercialize.

1.75 "FDA" means the U.S. Food and Drug Administration, and any successor entity thereto.

1.76 "Field" means the prevention, treatment, cure, diagnosis, prediction and detection of all diseases and conditions.

1.77 "First Commercial Sale" means, with respect to a Product and a country in the Territory, the first sale for end use or consumption of such Product in such country after all Regulatory Approvals and pricing and reimbursement approvals legally required for such sale have been granted by the applicable Regulatory Authority of such country or, if Regulatory Approval is not required, after the date on which sales are permitted by applicable Law.

1.78 "FTE" means one (1) person (or the equivalent of one (1) person) working full time for one (1) twelve (12) month period in a Development, regulatory or other relevant capacity (excluding persons employed in general and administrative, non-technical management or other non-technical capacities, and further excluding interns and co-operative education (co-op) employees) employed by Voyager or Neurocrine or any of their respective Affiliates and assigned to perform specified work, with such commitment of time and effort to constitute one (1) employee performing such work on a full-time basis, which for purposes hereof shall be [**] hours per year. No additional payment shall be made with respect to any person who works more than [**] hours per year (which person shall be deemed one (1) FTE) and any person who devotes less than [**] hours per year shall be treated as an FTE on a pro rata basis based upon the actual number of hours worked divided by [**] .

1.79 "FTE Costs" means the FTE Rate multiplied by the applicable number of FTEs who perform a specified activity pursuant to this Agreement.

1.80 "FTE Rate" means $[**] per FTE for the period commencing on the Effective Date and ending December 31, 2023. On January 1, 2024 and on January 1st of each subsequent Calendar Year, the foregoing rate shall be increased for the Calendar Year then commencing by the percentage increase, if any, in the Consumer Price Index ("CPI") as of December 31 of the then most recently completed Calendar Year with respect to the level of the CPI on December 31, 2023. Consumer Price Index or CPI means the Consumer Price Index – Urban Wage Earners and Clerical Workers, US City Average, All Items, 1982-84 = 100, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index).

1.81 "Future In-License Agreement" means any agreement between Voyager (or any of its Affiliates), on the one hand, and a Third-Party, on the other hand, entered into after the Effective Date, pursuant to which Voyager or any of its Affiliates acquires Control of any Know-How or Patent Right that, subject to Section 5.2, would be Voyager IP.

1.82 "GAAP" means United States Generally Accepted Accounting Principles consistently applied, as reported in the applicable financial statements.

1.83 "GBA1" means the gene that encodes glucosylceramidase beta 1 defined as Gene ID 2629.

1.84 "GBA1 Program" means all activities under this Agreement directed to the Development, Manufacture and Commercialization of Collaboration Candidates and Products directed to GBA1.

1.85 "GBA1 Program Development Plan" means the plan for Discovery Activities under the GBA1 Program and the budget for Voyager's activities under such plan, as such plan and budget may be approved or updated by the JSC from time to time in accordance with Section 2.1.2(a).

1.86 "GCP" means the then-current good clinical practice standards for clinical trials for pharmaceuticals, as set forth in the United States Food, Drug and Cosmetic Act, as amended from time to time, or other applicable law, and such standards of good clinical practice as are required

by the Regulatory Authorities of the EU and other organizations and Governmental Authorities in countries for which the applicable Product is intended to be Developed, to the extent such standards are not less stringent than United States GCP.

1.87 "Gene Therapy Product" means a virus, including an AAV, that: (a) comprises (i) a Capsid, (ii) a polynucleotide, whether single stranded or self-complementary, capable of selectively encoding one (1) or more payloads or including one (1) or more transgenes, and (iii) any other active or inactive components or ingredients; and (b) delivers such polynucleotide to certain cells of a patient for a purpose in the Field.

1.88 "GLP" means the then-current good laboratory practice standards promulgated or endorsed by the FDA as defined in 21 C.F.R. Part 58, or comparable regulatory standards in jurisdictions outside the United States.

1.89 "Governmental Authority" means any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

1.90 "HSR Act" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended from time to time.

1.91 "HSR Clearance Date" means the expiration or termination of all applicable waiting periods and requests for information (and any extensions thereof) under the HSR Act in the U.S.

1.92 "HSR Filing" means filings by Neurocrine and Voyager with the U.S. Federal Trade Commission and the Antitrust Division of the U.S. Department of Justice of a Notification and Report Form for Certain Mergers and Acquisitions (as that term is defined in the HSR Act) with respect to the matters set forth in this Agreement, together with all required documentary attachments thereto.

1.93 "Inbound Licensor" has the meaning set forth in Section 5.2.1.

1.94 "In-License Agreement" means: (a) any Existing In-License Agreement; and (b) any Future In-License Agreement.

1.95 "IND" means an investigational new drug application submitted to the FDA pursuant to Part 312 of Title 21 of the U.S. Code of Federal Regulations, including any amendments thereto. References herein to IND shall include any comparable filing(s) outside the U.S. for the investigation of any product in any other country or group of countries.

1.96 "Indemnified Party" has the meaning set forth in Section 13.3.

1.97 "Indemnifying Party" has the meaning set forth in Section 13.3.

1.98 "Indication" means a generally acknowledged disease or medical condition with respect to which at least one Clinical Trial is required by the FDA, EMA or PMDA to support

inclusion of such disease or medical condition in the indication statement of a package insert approved by such Regulatory Authority for a Product; provided that: (a) GBA1 Parkinson's disease and non-GBA1 Parkinson's disease shall be considered separate Indications; (b) except as set forth in subsection (a), prevention and treatment of the same disease or medical condition shall not be separate Indications; and (c) except as set forth in subsection (a) above, the treatment or prevention of the same disease or medical condition in different populations (e.g., adult and pediatric) shall not be separate Indications.

1.99 "Initiation" means, with respect to a Clinical Trial, the first dosing of the first subject enrolled in such Clinical Trial with a Product.

1.100 "Invention" means the result or act of invention (whether patentable or not) as determined in accordance with U.S. patent laws.

1.101 "Joint Arising IP" has the meaning set forth in Section 10.1.3(a)(ii)(C).

1.102 "Joint CMC Working Group" has the meaning set forth in Section 3.3.1(b).

1.103 "Joint Know-How" means any Know-How within the Joint Arising IP.

1.104 "Joint Patent Right" means any Patent Right within the Joint Arising IP.

1.105 "JRA Exception" has the meaning set forth in Section 15.14.

1.106 "JSC" has the meaning set forth in Section 3.1.1.

1.107 "Know-How" means all information, know-how and data, including trade secrets, Inventions (whether patentable or not), discoveries, methods, specifications, processes, expertise, technology, other non-clinical, pre-clinical and clinical data, documentation and results (including pharmacological, toxicological, biological, chemical, physical, safety and manufacturing data and results), analytical and quality control data and results, Regulatory Filings and other technical information. "Know-How" excludes any Patent Rights.

1.108 "Knowledge" means: (a) with respect to Voyager, the knowledge after reasonable investigation of the individuals set forth on Schedule 1.108; and (b) with respect to Neurocrine, the knowledge after reasonable investigation of the individuals set forth on Schedule 1.108, and including, in each case (a) and (b), if any such title role is no longer in existence, the knowledge after reasonable investigation of any individual having a similar role.

1.109 "Law" means any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.110 "Losses" has the meaning set forth in Section 13.1.

1.111 "[**]" means any of the following: [**].

1.112 "Major Market Countries" has the meaning set forth in Section 4.2.2.

1.113 "Manufacture" or "Manufacturing" means all activities related to the manufacturing of a Collaboration Candidate, Program Capsid or Product, including test method development and stability testing, formulation, process development, manufacturing scale-up, manufacturing for use in non-clinical and clinical studies, manufacturing for commercial sale, packaging, release of product, quality assurance/quality control development, quality control testing (including in-process, in-process release and stability testing) and release of product or any component or ingredient thereof, and regulatory activities related to all of the foregoing. "Manufacturing" may be included as part of Development, to the extent applicable.

1.114 "Milestone Event" has the meaning set forth in Section 8.2(a).

1.115 "Milestone Payment" has the meaning set forth in Section 8.2(a).

1.116 "Net Sales" means, with respect to any Product, the gross amount invoiced by Neurocrine, any of its Affiliates and or any Sublicensee (each, a "Selling Party") to a Third-Party (including a customer, distributor, wholesaler or end user) for sales of such Product, less the following deductions as calculated in accordance with the applicable Accounting Standard as consistently applied:

1.116.1 normal trade, cash, quantity and other customary discounts actually given to customers in the ordinary course of business;

1.116.2 rebates, credits and allowances given by reason of rejections, returns, damaged or defective product or recalls;

1.116.3 government-mandated rebates and any other compulsory payments, credits, adjustments and rebates actually paid or deducted;

1.116.4 price adjustments, allowances, credits, chargeback payments, discounts, rebates, fees and reimbursements or similar payments granted or made to managed care organizations, group purchasing organizations or other buying groups, pharmacy benefit management companies, health maintenance organizations and any other providers of health insurance coverage, health care organizations or other health care institutions (including hospitals), health care administrators, patient assistance or other similar programs, or to federal state/provincial, local and other governments, including their agencies, or to wholesalers, distributors or other trade customers;

1.116.5 reasonable and customary freight, shipping, insurance and other transportation expenses, if actually borne by the applicable Selling Party without reimbursement from any Third-Party;

1.116.6 reasonable distributors' and inventory management fees, including fees for services provided by wholesalers and warehousing chains, in connection with the sale and distribution of such Product;

1.116.7 that portion of administrative fees paid to group purchasing organizations, pharmacy benefit managers, Medicare prescription drug plans or any other facilitator of drug access for patients relating specifically to such Product;

1.116.8 uncollectible amounts or reasonable reserves accrued therefor (it being understood that any subsequent reductions in such accrual amounts due to collections in subsequent periods shall be included in Net Sales when such reductions occur);

1.116.9 that portion of the annual fee on prescription drug manufacturers imposed by the Patient Protection and Affordable Care Act, Pub. L. No. 111-148 (as amended) and reasonably allocable to sales of such Product;

1.116.10 sales, value-added, excise taxes, tariffs and duties, and other taxes and government charges directly related to the sale, delivery or use of such Product (but not including taxes assessed against the net income derived from such sale); and

1.116.11 any other similar and customary deductions that are consistent with GAAP, as agreed by the Parties in writing or, if the Parties fail to agree on any such deductions proposed by Neurocrine, as determined by a mutually agreed independent accounting expert, whose decision will be final and binding on the Parties.

If non-monetary consideration is received for any Product, Net Sales will be calculated based on the average price charged for such Product during the preceding Calendar Quarter in the relevant country, or in the absence of such sales, the fair market value of the Product, as determined by the Parties in good faith.

Resales or sales of a Product made in good faith between or among Neurocrine, any of its Affiliates or any Sublicensee shall not be included in the calculation of Net Sales as long as, with respect to such resales or sales, the first sale thereafter to a non-Sublicensee Third-Party is included in the calculation of Net Sales.

Net Sales shall not include any amounts received for Products supplied for use in clinical trials or supplied at or below the fully-burdened cost of goods thereof under early access, compassionate use, named patient, indigent access, patient assistance or other reduced pricing programs.

In the event that a Product under this Agreement is sold by a Selling Party in combination (a "Combination Product") with one or more therapeutically active compound(s) that are not Products ("Supplemental Ingredient(s)"), then "Net Sales" of the Combination Product shall be calculated using one of the following methods:

(x) By multiplying the Net Sales of the Combination Product (calculated prior to the application of this formula) by the fraction A/(A+B), where A is the average gross selling price, during the applicable Calendar Quarter in the country concerned, of the Product when sold separately, and B is the average gross selling price, during the applicable Calendar Quarter in the country concerned, of the Supplemental Ingredient(s) when sold separately; or

(y) In the event that no such separate sales are made of the Product or any of the Supplemental Ingredients in such Combination Product during the applicable Calendar Quarter in the country concerned, Net Sales shall be calculated using the above formula where A is the reasonably estimated commercial value of the Product sold separately and B is the reasonably estimated commercial value of the Supplemental Ingredient(s) sold separately. Any such estimates shall be determined using criteria to be mutually agreed upon by the Parties. If the Parties are unable to agree on the criteria for determining such estimates, the Parties will submit such dispute for resolution to a mutually agreed independent accounting expert, whose decision will be final and binding on the Parties.

1.117 "Neurocrine" has the meaning set forth in the Preamble.

1.118 "Neurocrine Background IP" has the meaning set forth in Section 10.1.1.

1.119 "Neurocrine IP" means the Neurocrine Know-How and the Neurocrine Patent Rights.

1.120 "Neurocrine Know-How" means: (a) all Know-How that (i) is Controlled by Neurocrine or any of its Affiliates on the Effective Date or during the Term, (ii) is disclosed or is required to be disclosed by or on behalf of Neurocrine to Voyager in connection with this Agreement, and (iii) is necessary or reasonably useful to Exploit in the Field in the Territory any Collaboration Candidate or Product; and (b) Neurocrine's interest in the Joint Know-How.

1.121 "Neurocrine Patent Rights" means: (a) all Patent Rights Controlled by Neurocrine or any of its Affiliates as of the Effective Date or during the Term that Cover any Collaboration Candidate or Product; and (b) Neurocrine's interest in the Joint Patent Rights.

1.122 "Neurocrine Plan" has the meaning set forth in Section 4.2.3.

1.123 "Neurocrine Product Marks" has the meaning set forth in Section 10.6.

1.124 "Neurocrine PRV Use" has the meaning set forth in Section 7.3.

1.125 "New Discovery Program" means all activities under this Agreement directed to the Development, Manufacture and Commercialization of Collaboration Candidates and Products directed to a particular New Discovery Target.

1.126 "New Discovery Program Development Plan" has the meaning set forth in Section 2.1.2(b).

1.127 "New Discovery Target" means each of the Targets listed in Schedule 1.127.

1.128 "Other Taxes" means custom, duties, sales tax, or other federal, state, local or foreign tax imposed on the provision of intangibles, goods, services, or similar tax.

1.129 "Out-of-Pocket Costs" means actual out-of-pocket costs and expenses paid by a Party or any of its Affiliates to Third Parties, including to a consultant, contractor, intern or co-operative education (co-op) employee of such Party.

1.130 "Party" and "Parties" has the meaning set forth in the Preamble.

1.131 "Patent Challenge" has the meaning set forth in Section 14.5.

1.132 "Patent Right" means: (a) any patent or patent application (including any provisional application) in any country or multinational jurisdiction in the Territory (including any converted application, continuation, continuation-in-part, continued prosecution application or divisional of any such application, any reissue, renewal, extension, substitution, reexamination, supplementary protection certificate, pediatric exclusivity period or the like of any such patent); (b) any foreign equivalent of any patent or patent application described in clause (a); and (c) all rights of priority in any of the foregoing.

1.133 "Payee" has the meaning set forth in Section 8.7.1.

1.134 "Payor" has the meaning set forth in Section 8.7.1.

1.135 "Person" means any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, Governmental Authority, or any other entity not specifically listed in this Section 1.135.

1.136 "Phase 1 Clinical Trial" means a human clinical trial (or a portion of a human clinical trial) of a product in any country, the principal purpose of which is a preliminary determination of safety in healthy individuals or patients, that would satisfy the requirements of 21 C.F.R. 312.21(a), or a similar clinical study prescribed by the relevant Regulatory Authorities in a country other than the United States.

1.137 "Phase 2 Clinical Trial" means a human clinical trial (or a portion of a human clinical trial) of a product in any country that would satisfy the requirements of 21 C.F.R. 312.21(b) and whose design is intended to explore a variety of doses, dose response, and duration of effect, and to generate initial evidence of clinical safety and activity in a target patient population, or a similar clinical study prescribed by the relevant Regulatory Authorities in a country other than the United States.

1.138 "Phase 3 Clinical Trial" means a human clinical trial (or a portion of a human clinical trial) of a product in any country that would satisfy the requirements of 21 C.F.R. 312.21(c) (or a similar clinical study prescribed by the relevant Regulatory Authorities in a country other than the United States) and whose design is intended to: (a) establish that the product is safe and efficacious for its intended use; (b) define warnings, precautions and adverse reactions that are associated with the product in the dosage range to be prescribed; and (c) support Regulatory Approval for such product.

1.139 "PHSA" means the Public Health Service Act as set forth in 42 U.S.C. Chapter 6A, as may be amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions and modifications thereto).

1.140 "Pivotal Clinical Trial" means a Clinical Trial that is designed to be sufficient to support the filing of a BLA for such product.

1.141 "PMDA" means the Pharmaceuticals and Medical Devices Agency in Japan, and any successor entity thereto.

1.142 "Potential Development Candidate" means a Collaboration Candidate selected by the JSC as a potential Development Candidate based on criteria established by the JSC and set forth in the applicable Development Plan.

1.143 "Pricing Approval" means such approval, agreement, determination or decision establishing the price for a Product that may be charged or reimbursed in any country or jurisdiction where a Governmental Authority or non-governmental pricing authority is required by Law to approve or determine the price or reimbursement of pharmaceutical or biological products.

1.144 "Product" means any product comprising a Collaboration Candidate, in any form, dose or formulation, and whether alone or in combination with other active or inactive ingredients. Except where the context otherwise requires, the term "Product" includes any Co-Co Product.

1.145 "Program" means the GBA1 Program or any New Discovery Program or Co-Co Program, but specifically excludes any Terminated Program. "Programs" means the GBA1 Program and all New Discovery Programs, but specifically excludes any Terminated Program.

1.146 "Program Capsid" means any Voyager Capsid that: (a) is included in a Collaboration Candidate; or (b) meets the Capsid profile criteria for a Program Target and is under consideration by the Parties for inclusion in a Collaboration Candidate.

1.147 "Program Capsid Patent Rights" means any Voyager Patent Right that: (a) Covers a Program Capsid; and (b) is not a Voyager Product-Specific Patent Right.

1.148 "Program Payload" means, on a Program-by-Program basis, a polynucleotide sequence, whether single stranded or self-complementary, that: (a) is intended to have a therapeutic effect on the applicable Program Target when packaged into a Voyager Capsid and delivered to the appropriate cells; and (b) is the subject of efforts under the Development Plan for such Program.

1.149 "Program Target" means, with respect to any Program, the Target that is the subject of such Program.

1.150 "Prosecution and Maintenance" or "Prosecute and Maintain" means, with regard to a Patent Right, the preparation, filing, prosecution and maintenance of such Patent Right, as well as re-examinations, reissues, appeals, and requests for patent term adjustments and patent term extensions with respect to such Patent Right. Notwithstanding anything to the contrary, "Prosecution and Maintenance" or "Prosecute and Maintain" shall not include any enforcement actions taken with respect to a Patent Right.

1.151 "PRV" has the meaning set forth in Section 7.3.

1.152 "PRV Sale" has the meaning set forth in Section 7.3.

1.153 "Public Official or Entity" means: (a) any officer, employee (including physicians, hospital administrators, or other healthcare professionals), agent, representative, department, agency, de facto official, representative, corporate entity, instrumentality or subdivision of any government, military or international governmental organization, including any ministry or department of health or any state-owned or affiliated company or hospital; or (b) any candidate for political office, any political party or any official of a political party.

1.154 "Receiving Party" has the meaning set forth in Section 11.1.

1.155 "Redacted Version" has the meaning set forth in Section 11.3.2.

1.156 "Regulatory Approval" means all approvals of the applicable Regulatory Authority necessary for the commercial marketing and sale of a product in a country(ies), excluding any pricing and reimbursement approvals that may be required.

1.157 "Regulatory Approval Application" means: (a) a BLA; or (b) any other application to seek Regulatory Approval of a product in any country or multinational jurisdiction, as defined in applicable Laws and filed with the relevant Regulatory Authorities of such country or jurisdiction.

1.158 "Regulatory Authority" means the FDA in the United States or any Governmental Authority in another country or regulatory jurisdiction in the Territory that is a counterpart to the FDA and holds responsibility for granting Regulatory Approval for a product in such country or regulatory jurisdiction, including the EMA and PMDA, and any successor(s) thereto.

1.159 "Regulatory Exclusivity" means any exclusive marketing rights or data exclusivity rights conferred by any Regulatory Authority with respect to any Product, excluding Patent Rights, that precludes the use of any clinical data collected and filed for such Product for the benefit of any Regulatory Approval for a generic or biosimilar product (for any use), including orphan or pediatric exclusivity where applicable.

1.160 "Regulatory Filing" means, with respect to a product, any documentation comprising any filing or application with any Regulatory Authority with respect to such product, or its use or potential use in the Field, any document submitted to any Regulatory Authority, including any IND and any Regulatory Approval Application, and any correspondence to, from or with any Regulatory Authority with respect to such product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

1.161 "Reimbursable Costs" has the meaning set forth in Section 4.1.1.

1.162 "Related Third-Party IP" has the meaning set forth in Section 5.2.2.

1.163 "Representatives" means a Party's or its Affiliate's officers, directors, employees, contractors, consultants, agents and other representatives.

1.164 "Royalty Term" has the meaning set forth in Section 8.4.

1.165 "Secondary Market Countries" has the meaning set forth in Section 4.2.2(b).

1.166 "Selling Party" has the meaning set forth in Section 1.116.

1.167 "Significant Safety Signal" means with respect to a Clinical Trial of a Product, that the results of such Clinical Trial indicate a safety finding relating to such Product that either: (a) is substantially irreversible or not monitorable in patients, e.g., neurodegeneration unrelated to pathology of the disease or death; or (b) results in Neurocrine's decision to designate such Product as a Terminated Product.

1.168 "Stock Purchase Agreement" has the meaning set forth in Section 8.1.2.

1.169 "Subcommittee" has the meaning set forth in Section 3.1.1.

1.170 "Sublicense" has the meaning set forth in Section 5.4.

1.171 "Sublicensee" has the meaning set forth in Section 5.4.

1.172 "Successful" means, with respect to a Clinical Trial for a Product, that: (a) the results of such Clinical Trial meet the pre-specified primary endpoint(s) set forth in the protocol for such Clinical Trial without a Significant Safety Signal; (b) Neurocrine (or its Affiliate or Sublicensee) advances a Product to the next stage of Development following completion of such Clinical Trial (irrespective of whether the results of such Clinical Trial meet the primary endpoint(s) set forth in the protocol therefor without a Significant Safety Signal); or (c) within [**] after complete readout of safety and efficacy data from such Clinical Trial (or such longer period as Neurocrine (or its Affiliate or Sublicensee) may reasonably determine in good faith is needed to assess such Clinical Trial results or a path for Development of such Product, or to receive or address feedback from a Regulatory Authority), (i) the condition in subsection (a) or (b) has not occurred, (ii) there is not any other Collaboration Candidate from such Program in Development, and (iii) such Program has not become a Terminated Program.

1.173 "Supplemental Ingredient(s)" has the meaning set forth in Section 1.116.

1.174 "Target" means a gene as defined by a specific gene ID, all mutants of such gene, derivatives or fragments with similar functional properties to such gene, or allelic variants of such gene: (a) whose DNA is delivered, replaced, substituted for, or altered upon administration of a Gene Therapy Product; (b) whose level of expressed RNA (including mRNA) or protein is modulated, silenced, augmented or eliminated upon administration of a Gene Therapy Product; or (c) whose protein expression product serves in whole or in part as an antigen and whereby, upon binding by an immunoglobulin encoded by a Gene Therapy Product such protein is neutralized or destroyed. All of the Gene Therapy Products described in the preceding clauses (a), (b) and (c) are considered "directed to" such Target.

1.175 "Term" has the meaning set forth in Section 14.1.

1.176 "Terminated Product" means any Collaboration Candidate or Product as to which this Agreement is terminated by the mutual agreement of the Parties or pursuant to ARTICLE 14. All Collaboration Candidates and Products in a Terminated Program are Terminated Products.

1.177 "Terminated Program" means a Program that is terminated by the JSC pursuant to Section 3.1.2(o), by the mutual agreement of the Parties or pursuant to ARTICLE 14.

1.178 "Territory" means all countries in the world.

1.179 "Third-Party" means any Person that is neither a Party nor an Affiliate of a Party.

1.180 "Title 11" has the meaning set forth in Section 5.7.

1.181 "Third-Party Claims" has the meaning set forth in Section 13.1.

1.182 "United States" or "U.S." means the United States of America and all of its territories and possessions.

1.183 "[**] License Agreement" means that certain non-exclusive license agreement, dated as of [**], by and between Voyager and [**].

1.184 "Valid Claim" means: (a) a claim of an issued and unexpired patent, which claim has not been revoked or held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction and that has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise; or (b) a claim of a patent application that has been filed and is being prosecuted in good faith and has been pending less than [**] from the date of filing of the earliest patent application from which such patent application claims priority, which claim has not been cancelled, withdrawn or abandoned or finally rejected by an administrative agency action from which no appeal can be taken.

1.185 "Voyager" has the meaning set forth in Preamble.

1.186 "Voyager Background IP" has the meaning set forth in Section 10.1.2.

1.187 "Voyager Capsid" means any Capsid that is used by Voyager in, or is made available by Voyager to Neurocrine for use in, any Program during the Discovery Period.

1.188 "Voyager IP" means the Voyager Know-How and Voyager Patent Rights.

1.189 "Voyager Know-How" means: (a) all Know-How that (i) is Controlled by Voyager or any of its Affiliates on the Effective Date or during the Term (other than through the grant of a license by Neurocrine), and (ii) is necessary or reasonably useful to Exploit any Collaboration Candidate or Product in the Field in the Territory; and (b) Voyager's interest in the Joint Know-How.

1.190 "Voyager Patent Rights" means: (a) all Patent Rights Controlled by Voyager or any of its Affiliates as of the Effective Date or during the Term that Cover any Collaboration Candidate or Product; and (b) Voyager's interest in the Joint Patent Rights. "Voyager Patent Rights" expressly exclude any Patent Rights licensed to Voyager under the license agreement by and between Voyager and [**] dated [**]. The Voyager Patent Rights expressly exclude any Patent Rights licensed to Voyager under the [**] License Agreement, which Patent Rights will not be

considered sublicensed hereunder unless and until Neurocrine requests in writing that such Patent Rights be so sublicensed following the naming of a Development Candidate with respect to the GBA1 Program or a New Discovery Program.

1.191 "Voyager Product-Specific Patent Rights" means any Voyager Patent Right with claims directed to: (a) the combination of a Voyager Capsid and a Program Payload; (b) any method of manufacture or use of such combination (and not only the Voyager Capsid therein); or (c) any modulation of a Program Target that is specific to such Program Target, its expression or the activity of its gene products. For clarity, any Voyager Patent Right that contains claims directed to a Voyager Capsid, which claims do not specifically recite a Program Payload or Program Target is not a Voyager Product-Specific Patent Right.

1.192 "Withholding Tax Action" has the meaning set forth in Section 8.11.3.

1.193 "Working Group" has the meaning set forth in Section 3.3.1.

ARTICLE 2
COLLABORATION; PRE-TRANSITION DEVELOPMENT

2.1 Collaboration and Programs.

2.1.1 Collaboration. The Parties agree to collaborate on the conduct of four (4) Programs under this Agreement: (a) the GBA1 Program; and (b) three (3) New Discovery Programs. The Development, Manufacturing and Commercialization activities for Collaboration Candidates and Products conducted pursuant to this Agreement under all four (4) Programs, as well as any such activities conducted pursuant to any Co-Co Agreement, together, shall constitute the "Collaboration".

2.1.2 Conduct of Programs.

(a) GBA1 Program. Voyager shall conduct Discovery Activities for the GBA1 Program pursuant to the GBA1 Program Development Plan. Promptly following the Effective Date, the Parties shall prepare the initial draft of the GBA1 Program Development Plan and submit it to the JSC for review and approval. The JSC shall approve the GBA1 Program Development Plan and Development Candidate Criteria for the GBA1 Program in accordance with Section 3.1.2(e) and Section 3.6.1. The JSC shall, prior to the end of each Calendar Year during the Discovery Period, review the GBA1 Program Development Plan and determine whether to update such plan, and shall prepare a detailed budget for Voyager's activities under the GBA1 Program Development Plan for the subsequent Calendar Year. A Party may also develop and submit to the JSC from time to time proposed substantive amendments to the GBA1 Program Development Plan. The JSC shall review such proposed amendments and may approve such proposed amendments or any other proposed amendments that the JSC may consider from time to time in its discretion and, upon any such approval by the JSC, the GBA1 Development Plan shall be deemed amended accordingly.

(b) New Discovery Programs. Voyager shall conduct Discovery Activities for the New Discovery Programs pursuant to a research plan and associated budget for

Voyager's activities (each such research plan, including the associated budget, a "New Discovery Program Development Plan"). Each New Discovery Program Development Plan shall set forth the activities to be conducted with respect to the applicable New Discovery Program during the Discovery Period. The Parties shall prepare the initial draft of each New Discovery Program Development Plan and submit it to the JSC for review and approval. The JSC shall approve each initial New Discovery Program Development Plan with respect to each New Discovery Program in accordance with Section 3.1.2(c). The JSC shall, prior to the end of each Calendar Year during the Discovery Period, review and update, as appropriate, each New Discovery Program Development Plan, including preparing a detailed budget for Voyager's activities under such New Discovery Program Development Plan for the subsequent Calendar Year. A Party may also develop and submit to the JSC from time to time proposed substantive amendments to any New Discovery Program Development Plan. The JSC shall review such proposed amendments and may approve such proposed amendments or any other proposed amendments that the JSC may consider from time to time in its discretion and, upon any such approval by the JSC, the applicable New Discovery Program Development Plan shall be amended accordingly.

(c) Target Replacement.

(i) Neurocrine shall have the right, [**] during the Discovery Period, to replace one New Discovery Target with a new CNS Target, subject to the availability of such new CNS Target as described below and further provided that, unless otherwise agreed by the Parties, any such new CNS Target may not be a Target for which modulation is reasonably believed (at the time the relevant Target replacement is being considered) to ameliorate [**]. Voyager will engage a Third-Party gatekeeper to review any replacement CNS Target proposed by Neurocrine to determine whether such proposed replacement CNS Target is (A) subject to any license, option, collaboration or similar obligations of Voyager to a Third-Party, or subject to *bona fide* negotiations with a Third Party seeking to obtain such rights, that would prevent Voyager from granting rights to Neurocrine with respect to such proposed CNS Target or (B) the subject of a current *bona fide* internal development program of Voyager for which Voyager has allocated budget or resources to be spent within the subsequent [**] period and appropriate for the applicable stage of the Development of such Program (each of (A) and (B), an "Occupied Target").

(ii) Voyager shall provide a list of Occupied Targets to the gatekeeper and shall require the gatekeeper (A) not to disclose Neurocrine's proposed replacement CNS Target to Voyager and (B) to notify the Parties of whether Neurocrine's proposed replacement CNS Target is an Occupied Target within [**] after Neurocrine discloses the proposed replacement CNS Target to the gatekeeper. If such proposed replacement CNS Target is an Occupied Target, then Neurocrine may continue to propose other CNS Targets until it proposes a CNS Target that is not an Occupied Target. If any proposed replacement CNS Target is not an Occupied Target, then such proposed CNS Target will become a New Discovery Target and Program Target, the replaced Target will no longer be a New Discovery Target or Program Target, and the JSC will promptly prepare a New Discovery Program Development Plan for the newly instituted New Discovery Target. If Neurocrine has not yet exhausted its right to replace one New Discovery Target with a new CNS Target and a CNS Target that was previously identified as an Occupied Target ceases to be an Occupied Target, Voyager shall update the gatekeeper with respect to the new status of such Target and, if Neurocrine had previously proposed such CNS

Target, the gatekeeper will notify Neurocrine that such CNS Target is no longer an Occupied Target.

(d) Limitation on Number of Potential Development Candidates and Development Candidates. There will be a maximum of four (4) Potential Development Candidates for which Development is being performed under any Program at any given time during the Discovery Period. If (i) a Potential Development Candidate (A) fails to meet Development Candidate Criteria established by the JSC and is removed from consideration to become a Development Candidate or (B) is named as a Development Candidate, then (ii) a new Potential Development Candidate may be Developed to replace the Potential Development Candidate that has failed or succeeded such that not more than four (4) Potential Development Candidates per Program are under consideration at any one time during the Discovery Period. Voyager will not be obligated to perform Development for any additional Collaboration Candidates in a Program after [**] Development Candidates have been named during the course of such Program unless otherwise agreed by the Parties.

2.1.3 Program Responsibilities. Each Party shall have the respective responsibilities assigned to it under the applicable Development Plan. The Parties shall conduct their respective Discovery Activities set forth in each Development Plan during the Discovery Period and shall use Commercially Reasonable Efforts to do so in accordance with the timelines and budgets therein.

2.1.4 Voyager Development Breach. If Voyager materially breaches its obligations with respect to the conduct of activities under any Development Plan (provided that, failure to achieve the Development Candidate Criteria shall not, in and of itself, be deemed a breach of Voyager's obligations under any Development Plan), then Neurocrine shall have the right but not the obligation, to elect the rights set forth in this Section 2.1.4 by written notice to Voyager (an "Assumption Notice"). Promptly following Voyager's receipt of the Assumption Notice, the Parties shall discuss the alleged breach and, if appropriate, a reasonable plan to cure such breach. If Voyager fails to cure such breach within (a) [**] after the Assumption Notice or (b) such longer period as may be reasonably required to cure the breach specified in the Assumption Notice (provided that Voyager is reasonably executing against a reasonable plan designed to cure such breach, which plan has been approved by Neurocrine in writing), then Neurocrine shall have the right, but not the obligation, to assume the conduct of the applicable Program, itself or through an Affiliate or Third Party contractor (other than a competitor of Voyager). If Neurocrine elects to assume the conduct of any Program, then Voyager shall conduct all activities and provide all assistance reasonably necessary to transition the Program to Neurocrine or its permitted designee, including the transfer of Voyager Know-How and the provision of materials. Notwithstanding anything to the contrary herein, in such event, Neurocrine shall not be responsible to reimburse any Development Costs incurred by Voyager in the relevant Program to conduct any activities that were not properly conducted by Voyager or whose conduct Neurocrine has assumed, but Neurocrine's obligations, including payment obligations to Voyager, under this Agreement will not otherwise be abrogated or modified.

2.1.5 Reporting Obligations. On a [**] basis until expiration of the Discovery Period, in advance of each regularly-scheduled JSC meeting, each Party that conducted Discovery Activities in such [**] shall provide the JSC with reasonably detailed reports describing the

activities undertaken and accomplishments achieved by such Party under each Development Plan, with Voyager setting forth the Development Costs incurred to conduct its activities and including a copy of all results generated (including all raw data) by Voyager in the performance of its activities under the Development Plan, in each case since the last such report. In addition, if Voyager engages any Third-Party subcontractor to conduct its Discovery Activities, Voyager shall provide the reports of any data or results received from the subcontractor within [**] of when such reports are received by Voyager. With respect to any material data generated by Voyager as a result of its Discovery Activities, Voyager shall provide such data (including all supporting raw data) to Neurocrine within [**] after Voyager's internal reporting of such data, even if such data are only preliminary (non-final). Voyager shall promptly respond to Neurocrine's reasonable requests for more information with respect to reporting of activities and Development Costs incurred in each [**] report with respect to any Program. In addition, at Neurocrine's request in between such [**] reports, Voyager shall provide all information reasonably requested by Neurocrine, including results and Development Costs incurred. On an [**] basis and Program-by-Program basis following the end of the Discovery Period, in advance of the regularly scheduled JSC meeting, Neurocrine shall provide Voyager with a reasonably detailed report describing the activities undertaken and accomplishments achieved under each Program, including a summary of all results generated by Neurocrine under each Program, in each case since the last such report. Neurocrine shall promptly respond to Voyager's reasonable requests for more information with respect to each such report with respect to any Program.

2.1.6 Development Candidates. Either Party may notify the JSC of any Potential Development Candidate or other Collaboration Candidate that it desires to nominate as a Development Candidate. In such event, the JSC will determine whether such nominated Potential Development Candidate or other Collaboration Candidate meets the Development Candidate Criteria. Each of the Parties shall respond to reasonable requests from the JSC for additional information regarding each Potential Development Candidate or other Collaboration Candidate nominated as a Development Candidate, including as to whether the Voyager Capsid therein is subject to any Third-Party rights. If the JSC agrees that a Potential Development Candidate or other Collaboration Candidate meets the Development Candidate Criteria, or if the JSC otherwise decides to designate such Potential Development Candidate or other Collaboration Candidate as a Development Candidate notwithstanding its failure to achieve the Development Candidate Criteria, then such Potential Development Candidate or other Collaboration Candidate shall thereafter be deemed to be a Development Candidate hereunder.

2.1.7 Safety Data Sharing. For any Program Capsid that is included in a Product and also in a product that is not a Product, the Parties will, upon either Party's reasonable request, negotiate reasonably in good faith to enter into a pharmacovigilance agreement providing for the exchange of safety data related to such Program Capsid, including as necessary for each Party to comply with its regulatory reporting obligations.

2.1.8 Voyager Capsid Exclusivity.

(a) If Voyager identifies during the Discovery Period, [**] an Exclusivity-Eligible Capsid [**], then Voyager shall promptly notify Neurocrine. For purposes of this Section 2.1.8, a Capsid is an "Exclusivity-Eligible Capsid" if such Capsid [**] (each, an "Exclusivity-Eligible Capsid"). In addition, Voyager may designate [**]. For clarity, none of the

following will be deemed to be Exclusivity-Eligible Capsids without Voyager's prior written consent (in Voyager's sole discretion): [**].

(b) Subject to the exclusions set forth in Section 2.1.8(a), Neurocrine shall have the right during the Discovery Period to select [**] to be subject to the rights described below in this Section 2.1.8(b) (each, an "Exclusive Capsid"), which Capsid may be used for any or all Programs, by written notice to Voyager given within [**] after the notice described in Section 2.1.8(a) above; provided, however, that no more than [**] may be designated by Neurocrine as an Exclusive Capsid. Subject to the foregoing, Neurocrine may elect to change its Exclusive Capsid designation for any Program at any time during the Discovery Period to a different Capsid that is an Exclusivity-Eligible Capsid at the time of such change in designation. Neither Voyager nor any of its Affiliates shall, without Neurocrine's prior written consent, either alone or with or for any Third-Party, Develop (except that Voyager or its Affiliate may, prior to the existence of a Development Candidate containing an Exclusive Capsid, conduct basic scientific, non-clinical and pre-clinical Development with respect to the biological mechanism of action, pharmacology, structure-activity relationship (SAR) or the like for any Gene Therapy Product containing such Exclusive Capsid that is not directed to a Program Target), Manufacture or Commercialize any product that includes an Exclusive Capsid or grant any Affiliate or Third-Party a license or sublicense to enable any Third-Party to do so. Notwithstanding anything to the contrary herein, any [**].

(c) Notwithstanding Section 2.1.8(b), if Neurocrine ceases to apply Commercially Reasonable Efforts to Develop or Commercialize a Collaboration Candidate, Development Candidate or Product incorporating a particular Exclusive Capsid, then such Capsid will cease to be an Exclusive Capsid hereunder, and Voyager will no longer be subject to the obligations set forth in Section 2.1.8(b) above with respect to such Capsid.

2.2 Development Costs.

2.2.1 In General. Neurocrine shall be responsible for all Development Costs incurred by Voyager in connection with Voyager's performance under each applicable Development Plan in accordance with the terms of this Agreement, provided that such Development Costs are in accordance with the budget set forth in such Development Plan, subject to Section 2.2.2.

2.2.2 Payment. Within [**] following the end of each Calendar Quarter in which Voyager conducts activities under a Development Plan, Voyager shall provide Neurocrine with a preliminary report detailing, on a Program-by-Program basis, all Development Costs incurred by Voyager in such Calendar Quarter to conduct its activities under each Development Plan in accordance with the budget in such Development Plan, such report to list the name, title and function of each individual conducting Discovery Activities and the number of hours worked by each such individual on each Program. Within [**] following the end of each Calendar Quarter in which Voyager conducts activities under a Development Plan, Voyager shall provide Neurocrine with an invoice detailing, on a Program-by-Program basis, all Development Costs actually incurred by Voyager in such Calendar Quarter to conduct its activities under each Development Plan in accordance with the budget in such Development Plan, such invoice to list the name, title and function of each individual conducting Discovery Activities and the number of hours worked

by each such individual on each Program. Voyager shall include with each invoice documentation for any individual Out-of-Pocket Costs in excess of [**] Dollars ($[**]). To the extent that the invoiced amounts for each activity are less than or equal to [**] percent ([**]%) of the corresponding amounts for such activity set forth in the budget in the applicable Development Plan, Neurocrine shall pay each such invoice, unless subject to a bona fide dispute, within [**] after receipt thereof. Voyager shall maintain detailed records of its Development Costs pursuant to Section 8.7.1, including with respect to the number of hours spent by each individual on each Program. Neurocrine shall have the right to conduct an audit of such books and records of Voyager to verify the amount of Development Costs and the accuracy of reports and invoices provided under this Section 2.2.2 pursuant to Section 8.7. Such audit shall not be performed more frequently than [**] period, unless an audit in the prior [**] period shows that Voyager has overcharged Neurocrine by more than [**] percent ([**]%), in which event Neurocrine shall be entitled to audit [**] for a period of [**] thereafter. If Voyager anticipates that the FTE Costs or Out-of-Pocket Costs it incurs to conduct any activity under a Development Plan will exceed, or if any such costs do exceed, the amount set forth in the applicable budget for such activity by more than [**] percent ([**]%), Voyager shall promptly notify the JSC, and the JSC shall discuss in good faith and decide whether to increase such budget.

ARTICLE 3
MANAGEMENT OF THE COLLABORATION

3.1 Joint Steering Committee and Subcommittees.

3.1.1 The Parties hereby establish the Joint Steering Committee (the "JSC"), which will be the same committee as the JSC (as defined in the 2019 CLA) under the 2019 CLA, to serve as (a) the oversight and decision-making body for the Discovery Activities and activities under the Co-Co Agreement to be conducted by the Parties pursuant to this Agreement and (b) a forum for information exchange and discussion with respect to all other activities under this Agreement, in each case (a) and (b) as more fully described in this ARTICLE 3. The Parties anticipate that the JSC will not be involved in day-to-day implementation of the activities under this Agreement but shall have the responsibilities and decision-making authority set forth herein or as mutually agreed by the Parties in writing from time to time. The JSC may establish subcommittees as set forth in Section 3.2 (each a "Subcommittee").

3.1.2 Responsibilities. The JSC shall perform the following functions with respect to the Collaboration, subject to the final decision-making authority of the respective Parties as set forth in Section 3.6:

(a) serve as an information transfer vehicle to facilitate discussions regarding the Development of Collaboration Candidates;

(b) review and determine whether to update the GBA1 Program Development Plan or New Discovery Program Development Plans (including related budgets) at the end of each Calendar Year, or at other times, in accordance with Sections 2.1.2(a) and 2.1.2(b);

(c) within [**] after submission pursuant to Section 2.1.2(b), review, provide comments on and approve each New Discovery Program Development Plan;

(d) review and approve any substantive amendments to a Development Plan proposed by a Party, including any amendments to the budget therein;

(e) establish the GBA1 Program Development Plan and the criteria for Potential Development Candidates and Development Candidate Criteria for the GBA1 Program within [**] after the Effective Date;

(f) establish the criteria for Potential Development Candidates and Development Candidate Criteria for each New Discovery Program promptly after approval of the Development Plan therefor, and in any event within [**] after the Effective Date;

(g) determine whether to select a Collaboration Candidate as a Potential Development Candidate;

(h) review and approve the designation of each Development Candidate in accordance with Section 2.1.6;

(i) review and discuss progress reports on the Development activities submitted by each Party, including the reports submitted under Section 2.1.4 and Section 4.2.4;

(j) address any issues or disputes arising from the conduct of the Discovery Activities hereunder;

(k) review and approve plans for co-Development and co-Commercialization in accordance with the Co-Co Agreement entered into by the Parties;

(l) after completion of the applicable Discovery Activities and until the Co-Co Trigger Date, review and approve the Neurocrine Plan for the GBA1 Program and review (but not approve) the Neurocrine Plan for the New Discovery Programs and for the GBA1 Program if the Co-Co Option expires without Voyager's exercise thereof;

(m) at the Co-Co Trigger Date, review and discuss Voyager's capabilities for conducting activities under a Co-Co Agreement;

(n) resolve disputes between the Parties with respect to the Co-Co Program;

(o) determine that successful Development under a Development Plan is not commercially or scientifically viable, and terminate such Program, thereby deeming such program a Terminated Program;

(p) review and discuss Product formulation and formulation optimization;

(q) periodically review and provide comments on the Development and post-approval status of each Product;

(r) review and discuss manufacturing scale-up, validation and Product supply;

(s) review and discuss any potential Future In-License Agreements;

(t) review and discuss any reports or recommendations of the Collaboration IP Working Group, the Publication Working Group, the Joint CMC Working Group or any other Subcommittee or Working Group;

(u) resolve any disputes of the Collaboration IP Working Group, the Publication Working Group, the Joint CMC Working Group or any other Subcommittee or Working Group;

(v) review and approve each [**] publication plan presented by the Publication Working Group;

(w) form such Subcommittees and additional Working Groups as it deems necessary to achieve the objectives and intent of this Agreement; and

(x) perform such other responsibilities as may be assigned to the JSC pursuant to this Agreement or as may be mutually agreed upon by the Parties in writing from time to time.

Except with respect to Co-Co Products in the Co-Co Territory as set forth in Section 4.1.2(a), the JSC will not have any decision-making authority with respect to Development of Products outside of the Discovery Activities or the Commercialization of Products, including the content of the Neurocrine Plans. Notwithstanding anything to the contrary, the JSC shall not have any authority beyond the specific matters set forth in this Section 3.1.2, and in particular shall not have any power to amend or modify the terms of this Agreement or waive a Party's compliance with this Agreement.

3.2 Formation and Dissolution of Subcommittee(s). The JSC may, in its discretion, establish Subcommittees from time to time to handle specific matters within the scope of the JSC's area of authority and responsibility, and no Subcommittee's authority and responsibility may be greater than that of the JSC itself. Each Subcommittee shall have such authority and responsibility as determined by the JSC from time to time, and decisions and recommendations of any Subcommittee shall be made in accordance with Section 3.6. The JSC shall determine when each Subcommittee it forms shall be dissolved.

3.3 Working Groups.

3.3.1 Formation of Working Groups. From time to time, the Parties, the JSC or any Subcommittee (each, a "Committee") may establish a working group (each, a "Working Group") to oversee particular projects or activities (e.g., a research and development working group). Each Working Group shall undertake the activities allocated to it herein or delegated to it by the Committee to which it reports. During the process of establishing a Working Group, such Working Group and the Committee to which it reports shall agree regarding which matters such

Working Group will resolve on its own and which matters such Working Group will advise the Committee regarding (and with respect to which such advice-specific matters the Committee will resolve); provided that the Parties acknowledge and agree that each Working Group is intended to function primarily in a supporting role providing advice to the Committee to which it reports, but that each Working Group will be best positioned to provide expedited guidance and decisions regarding certain operational matters as determined by the Committee to which such Working Group reports.

(a) Collaboration IP Working Group. The Parties shall establish an intellectual property working group (the "Collaboration IP Working Group") within [**] following the Effective Date. The Collaboration IP Working Group will be responsible for providing the JSC and the Parties with guidance with respect to matters relating to (i) the preparation, filing, prosecution and maintenance of Voyager Patent Rights and Joint Patent Rights, including patent term extensions, (ii) freedom-to-operate matters, (iii) discussing any challenges to any Third-Party's Patent Rights that may Cover any Collaboration Candidate, and (iv) advising the JSC regarding which of the Existing In-License Agreements are relevant to any Collaboration Candidate. The Collaboration IP Working Group will report to the JSC.

(b) Publication Working Group. The Parties shall establish a publication working group (the "Publication Working Group") within [**] following the Effective Date. The Publication Working Group will include the members of the Collaboration IP Working Group and will be responsible for preparing and providing to the JSC, on a [**] basis, a [**] plan for publications related to the Programs, Collaboration Candidates and Products, for the JSC's review and approval. The Publication Working Group will report to the JSC.

(c) Joint CMC Working Group. The Parties shall establish a joint Manufacturing working group (the "Joint CMC Working Group") within [**] following the Effective Date. The Joint CMC Working Group will be responsible for providing the JSC and the Parties with guidance with respect to matters relating to the generation and maintenance of chemistry, manufacturing and controls (CMC) data required by applicable Law to be included or referenced in, or otherwise support, an IND or Regulatory Approval Application and coordinating the sharing and exchange of such data between Voyager and Neurocrine. The Joint CMC Working Group will report to the JSC.

3.4 Membership. Each Committee shall be composed of an equal number of representatives appointed by each of Voyager and Neurocrine. The JSC shall be comprised of [**] representatives of each Party, and each other Committee shall be comprised of such number of representatives of each Party as is agreed upon by the Parties. Each Party shall appoint at least one (1) representative to each Working Group and shall have the right, but not the obligation, to appoint the same number of representatives to any Working Group as are appointed by the other Party to such Working Group. Each individual appointed by a Party as a representative to the JSC shall be an employee of such Party. Each individual appointed by a Party as a representative to any Subcommittee or Working Group shall be an employee of such Party, an employee of such Party's Affiliate or, upon the other Party's approval, a contractor to such Party or its Affiliate; provided that, with respect to the Collaboration IP Working Group, either Party may appoint outside intellectual property counsel as a representative without such approval. Each Party may replace any of its Committee or Working Group representatives at any time upon written notice to the

other Party, which notice may be given by e-mail sent to the other Party's co-chairperson of such Committee and, with respect to a change of representatives to any Working Group, to the other Party's co-chairperson of the Committee to which such Working Group reports. Each Committee and Working Group shall be co-chaired by one designated representative of each Party. Any member of a Committee or Working Group may designate a substitute who is an employee of the applicable Party to attend and perform the functions of that member at any meeting of such Committee, as applicable. Notwithstanding the foregoing, each Party shall ensure at all times during the existence of a Committee or Working Group that its representatives (including any replacements or substitutes therefor) on such Committee or Working Group are appropriate in terms of seniority, experience, expertise and decision-making authority and are subject to obligations of confidentiality and non-use with respect to the other Party's Confidential Information that are no less stringent than those set forth in ARTICLE 11.

3.5 Meetings.

3.5.1 The co-chairpersons shall be responsible, with respect to their Committee or Working Group, as applicable, for: (a) calling meetings; (b) preparing and circulating an agenda in advance of each meeting; provided that the co-chairpersons shall include any agenda items proposed by either Party on such agenda; (c) ensuring that all decision-making is carried out in accordance with the voting and dispute resolution mechanisms set forth in this Agreement; and (d) preparing and issuing minutes of each meeting within [**] (or such shorter time as is agreed by the relevant Committee or Working Group) thereafter. The location of regularly scheduled meetings shall alternate between Voyager's offices located in Cambridge, Massachusetts and Neurocrine's offices located in San Diego, California, unless otherwise agreed by such Committee or Working Group. Such Committee or Working Group may also determine that a meeting will instead be held telephonically, by video conference or by any other media; provided, however, that the JSC shall hold at least [**] in person each Calendar Year, unless the Parties mutually agree otherwise. Each Party may designate the same individual as a representative on more than one Committee or Working Group. Each Party will bear all expenses it incurs in regard to participating in all meetings of each Subcommittee and Working Group, including all travel and lodging expenses.

3.5.2 The JSC shall meet [**] during the Discovery Period and thereafter at least [**] prior to the First Commercial Sale of a Product from all Programs, and [**] thereafter, or more or less frequently as the Parties mutually deem appropriate, on such dates and at such places and times as provided herein or as the Parties shall agree. Notwithstanding the foregoing, the JSC shall continue to meet [**] after expiration of the Discovery Period until [**] after the Co-Co Trigger Date (and if Voyager exercises the Co-Co Option, thereafter until entry into the Co-Co Agreement that provides new parameters) to discuss the GBA1 Program.

3.6 Decision-Making.

3.6.1 Escalation to JSC. Except as otherwise provided herein, all decisions of each Committee and each Working Group shall be made by consensus, with all of a Party's voting members collectively having one (1) vote. If a Committee or Working Group other than the JSC is incapable of reaching unanimous agreement on a matter before it within [**] after first attempting to decide such matter, the matter shall be referred to the JSC for resolution. Unless the

Parties mutually agree otherwise, the JSC shall attempt to reach unanimous agreement on any matter within the scope of its authority within [**] after first attempting to decide such matter and after having at least [**]. If the JSC does not resolve such matter as set forth in this Section 3.6.1, then either Party may escalate the matter to the Executive Officers for resolution in accordance with Section 3.6.2. Notwithstanding the foregoing, (a) matters in dispute for which greater exigency is required, may be escalated more quickly as agreed by the Parties and (b) in the event of a dispute regarding the establishment of the GBA1 Program Development Plan or the Development Candidate Criteria under Section 3.1.2(e) for the GBA1 Program, such dispute shall be resolved by the Chief Executive Officer of Voyager and the Chief Scientific Officer of Neurocrine (or, in the event of a change of personnel, appropriate designees), and shall not be subject to Section 3.6.3 or any further escalation under Sections 15.2 or 15.3.

3.6.2 Escalation to the Executive Officers. The Parties' respective Executive Officers shall meet within [**] after a matter within the scope of the JSC's authority is referred to them for resolution pursuant to the last sentence of Section 3.6.1, and shall negotiate in good faith to attempt to resolve the matter. If the Executive Officers are unable to resolve such matter within [**] after the matter is referred to them, then the matter will be determined in accordance with Section 3.6.3.

3.6.3 Final Decision Making Authority. With respect to any matter within the scope of the JSC's authority that remains unresolved after escalation to the Executive Officers under Section 3.6.2, the matter will be finally resolved as set forth below.

(a) GBA1 Program. Subject to the limitations set forth in Section 3.6.3(c), with respect to the GBA1 Program, such matters will be resolved as follows:

(i) Prior to the exercise by Voyager of the Co-Co Option for the GBA1 Program, or if Voyager does not exercise the Co-Co Option, Neurocrine shall have the right to finally decide any unresolved matter relating to the GBA1 Program that is within the scope of the JSC's authority except with respect to the establishment of the GBA1 Program Development Plan or the determination of the Development Candidate Criteria for the GBA1 Program as set forth in Section 3.6.1; and

(ii) From and after the timely exercise by Voyager of its Co-Co Option for the GBA1 Program: (A) to the extent any unresolved matter within the scope of the JSC's authority relates to the Development or Manufacturing of Co-Co Products for the Co-Co Territory prior to commercial launch of any Co-Co Product in the Co-Co Territory, neither Party shall have the right to decide such unresolved matter and such unresolved matter shall be deadlocked until resolved by mutual agreement of the Parties or the JSC; and (B) to the extent any unresolved matter within the scope of the JSC's authority relates to the Development or Manufacturing of Co-Co Products following commercial launch of any Co-Co Product in the Co-Co Territory, or relates to Commercialization of Co-Co Products, or relates to the Development outside of the Co-Co Territory of Products in the GBA1 Program, Neurocrine shall have the right to decide such unresolved matter, subject to Section 4.1.2(a).

(b) New Discovery Programs. Subject to the limitations set forth in Section 3.6.3(c), Neurocrine will have the right to finally decide any unresolved matters relating to the New Discovery Programs that is within the scope of the JSC's authority.

(c) Limitations on Scope and Final Decision Making Authority. In no event shall any Committee, Working Group or any Party alone have the power or authority to: (i) amend this Agreement; (ii) determine whether a Party has fulfilled or breached its obligations under this Agreement; (iii) impose any requirements on either Party to undertake obligations beyond those for which it is responsible, or to forgo any of its rights, under this Agreement; (iv) make a decision that is expressly stated under this Agreement to require the mutual agreement of the Parties or of the JSC; (v) make a decision that could reasonably be expected to cause Voyager to breach an In-License Agreement or give rise to the right of the applicable Inbound Licensor to take any action under such In-License Agreement; or (vi) require any Party to perform any act that it reasonably believes to be inconsistent with any Law. In addition, Neurocrine will not have the right to exercise any final decision making authority to: (x) [**]; or (y) [**]. Any decision made by the Executive Officers in accordance with Section 3.6.2 or by a Party in accordance with this Section 3.6.3 shall be considered a decision made by the JSC.

3.7 Alliance Managers. Each Party's alliance manager under the 2019 CLA will act as alliance manager for such Party under this Agreement (each, an "Alliance Manager"). Each Alliance Manager shall be permitted to attend meetings of the JSC as a nonvoting observer, subject to the confidentiality provisions of ARTICLE 11. The Alliance Managers shall be the primary point of contact for the Parties regarding the activities contemplated by this Agreement. The Alliance Managers shall also be responsible for assisting the JSC in performing its oversight responsibilities. The name and contact information for each Party's Alliance Manager, as well as any replacement chosen by such Party, in its sole discretion, from time to time, shall be promptly provided to the other Party in accordance with Section 15.8.

3.8 Authority. Each Party will retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers or discretion will be delegated to or vested in the JSC or any other Subcommittee or any Working Group unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

ARTICLE 4
POST-DISCOVERY ACTIVITIES

4.1 Co-Development and Co-Commercialization.

4.1.1 Voyager's Opt-In Right. Voyager shall have the right to elect to co-Develop and co-Commercialize Products that are the subject of the GBA1 Program in the United States (the "Co-Co Option") by providing Neurocrine with written notice of such election within [**] following the Co-Co Trigger Date. Upon such exercise, the Parties shall negotiate in good faith and enter into an agreement, which shall be based on terms and conditions substantially the same as those set forth in this Section 4.1 and otherwise consistent with this Agreement (each such agreement, a "Co-Co Agreement"), pursuant to which the Parties will jointly Develop and Commercialize and share equally in the Development Costs, Commercialization costs and profit or loss resulting from the Development and Commercialization of such Products in the United

States (the "Co-Co Territory"). Once Voyager exercises the Co-Co Option, each Product in the GBA1 Program shall be designated a "Co-Co Product" hereunder and the GBA1 Program shall be designated the "Co-Co Program" hereunder, the Parties will share equally in United States Development Costs incurred thereafter, and Voyager will reimburse Neurocrine, as described in Sections 4.1.2(d), 4.1.3 and 7.3, for fifty percent (50%) of all Development Costs incurred by Neurocrine in connection with the Development of Products in the GBA1 Program prior to Voyager's exercise of the Co-Co Option (the "Reimbursable Costs"). The "Co-Co Trigger Date" shall mean the date on which Voyager receives topline data from the first Phase 1 Clinical Trial for a Product that is the subject of the GBA1 Program. Following the first Change of Control of Voyager, Voyager will be obligated to pay interest on the Reimbursable Costs equal to [**]% per annum (paid by the same mechanism as the Reimbursable Costs), provided that Voyager may instead pay all of the Reimbursable Costs, without interest, within [**] after the later of (i) Voyager's exercise of the Co-Co Option and (ii) effectiveness of such Change of Control.

4.1.2 Co-Co Agreement General Principles. It is the intent of the Parties that Development and Commercialization of each Co-Co Product in the Co-Co Territory under the applicable Co-Co Agreement will be conducted in accordance with the following principles, except as otherwise mutually agreed by the Parties in writing. The Parties shall take into account and attempt to implement the following principles in their decision-making, including preparation, review and approval of any updates to and amendments of the Development plan and Commercialization plan under such Co-Co Agreement:

(a) Development and Commercialization of each Co-Co Product in and for the Co-Co Territory shall be conducted according to a mutually agreed Development plan and Commercialization plan, respectively, prepared and updated periodically by Neurocrine, in consultation with Voyager, and submitted to the JSC for review and approval. Such plans shall (i) set forth the Development activities and Commercialization activities, respectively, to be undertaken by the Parties with respect to the applicable Co-Co Product in and for the Co-Co Territory in the subsequent [**], (ii) be updated at least [**] and (iii) include a related detailed budget. Either Party may propose amendments to a Development plan or Commercialization plan to the JSC for review and approval. No Development or Commercialization activities shall be delegated to a Party in the Development plan or Commercialization plan (or any amendment thereto) without such Party's prior agreement. Each Party will use Commercially Reasonable Efforts to perform the Development and Commercialization activities delegated to such Party in the Development plan and Commercialization plan, as applicable. Each Party's Development Costs for the Co-Co Program shall be calculated in a manner consistent with Development Costs calculation under this Agreement (including related definitions). FTE Costs with respect to Commercialization costs for the Co-Co Program shall be calculated in a manner consistent with this Agreement. Notwithstanding the foregoing, the terms of the Co-Co Agreement (i) shall not require any realignment or decrease in the size of the then-current Neurocrine field forces, and (ii) shall be reasonably directed to maximize profit from the Co-Co Product.

(b) The Development plan and the Commercialization plan under the Co-Co Agreement shall each include an allocation of responsibilities between the Parties, including Development, sales, marketing and promotional efforts (including the number and nature of representatives and their geographical alignment, compensation structure and sales force hiring

plans), reasonably and equitably determined after taking into consideration each Party's expertise, capabilities, staffing and then-existing resources to take on such activities. Notwithstanding the foregoing, but subject to the last sentence of Section 4.1.2(a), the Development plan and the Commercialization plan under the Co-Co Agreement shall include, if reasonably requested by Voyager, meaningful participation in Development activities, Commercialization activities (including participation in field sales and detailing), preparation for Commercialization, and medical affairs activities by Voyager, provided that in all cases Neurocrine will be responsible for booking sales of Co-Co Products.

(c) The Parties shall share equally in Development and Commercialization costs incurred by either Party or its Affiliates in accordance with the applicable budgets in conducting activities for the Co-Co Territory in accordance with the Development plan and Commercialization plan under the Co-Co Agreement. The Co-Co Agreement shall provide that (i) if either Party incurs Development Costs or Commercialization costs in excess of [**] percent ([**]%) of the Development Costs or Commercialization costs, as applicable, budgeted for activities assigned to such Party in the budget of the then-current version of the Development plan or Commercialization plan, as applicable, then such Party shall be solely responsible for such excess costs unless such Party has received the other Party's written approval to share such excess costs and (ii) global Development Costs incurred for Development activities that support Regulatory Approval in the Co-Co Territory and in other countries of the Territory shall be reasonably and equitably allocated to the Co-Co Program in accordance with the reasonably expected proportion of Co-Co Product sales in the Co-Co Territory as compared with other countries in the Territory, as mutually agreed by the Parties (it being understood that Development Costs incurred for activities conducted outside the Co-Co Territory that are solely for supporting Regulatory Approval in the Co-Co Territory will be fully included in the shared Development Costs without allocation).

(d) All profit or loss (which shall be defined in the Co-Co Agreement in a customary manner) and any amounts paid to any Inbound Licensor under an In-License Agreement from and after the exercise of the Co-Co Option (including royalty, milestone, and sublicense income payments) with respect to the Co-Co Products shall be shared between the Parties equally and as such amounts are reasonably determined by the Parties to be allocable to the Co-Co Territory; provided that fifty percent (50%) of Voyager's profit share shall be paid to Neurocrine until the aggregate of such amount equals the Reimbursable Costs. Proceeds of the sale of any PRV granted to Neurocrine in connection with the approval of the BLA for a Co-Co Product shall be considered Net Sales for the Co-Co Program and costs and expenses associated with any Third-Party engaged to facilitate such sale shall be considered a cost for the Co-Co Program, but only if the JSC approves the engagement of such Third-Party prior to such sale. Regardless of the Parties' respective insurance coverages, any losses incurred by either Party arising from Third-Party Claims related to Exploitation of the Co-Co Products in or for the Co-Co Territory, including Third-Party Claims based on intellectual property infringement, product liability or personal injury, shall be shared equally between the Parties, except to the extent resulting from the gross negligence, recklessness or intentional misconduct of a Party or any of its Affiliates or its or their respective Representatives or a Party's breach of this Agreement.

(e) Neurocrine's obligation to pay the royalty set forth in Section 8.3.1(a) shall terminate, and Neurocrine's obligation to make milestone payments with respect to such Co-Co Products shall be modified as set forth in Section 8.2(b).

(f) Regardless of the specific division of responsibility between the Parties for particular activities at any particular time, the JSC shall serve as a conduit for sharing information, knowledge and expertise relating to the Development and Commercialization of each Co-Co Product.

(g) The Co-Co Agreement shall specify that the mutual consent of both Parties shall be required to Develop and Commercialize each Co-Co Product with any Third-Party in the Co-Co Territory, including the sale, licensing or divestiture of marketing rights or product assets as to such Co-Co Product in the Co-Co Territory.

(h) The dispute resolution provisions in the Co-Co Agreement shall mirror Sections 15.2 and 15.3 of this Agreement and the Parties shall agree that any arbitration brought under a Co-Co Agreement may be consolidated with an arbitration brought under this Agreement.

4.1.3 Cost and Profit Sharing. Each Party shall receive (in the case of profits) or pay (in the case of losses), as applicable, its allocable share of profit and losses with respect to each Co-Co Product in the Co-Co Territory. The Parties shall share equally in such profit and losses with Neurocrine entitled to or responsible for 50% of profits and losses and Voyager entitled to or responsible for 50% of profits and losses with respect to each Co-Co Product in the Co-Co Territory. Notwithstanding the foregoing, Neurocrine will receive (in addition to Neurocrine's 50% share of profit) 50% of Voyager's share of profit until the aggregate of such amount equals the Reimbursable Costs (i.e., Neurocrine will receive 75% of profit and Voyager will receive 25% of profit until the aggregate of 25% of profit equals the Reimbursable Costs).

4.1.4 Termination of Co-Co Agreement.

(a) Voyager shall have the right to terminate the Co-Co Agreement for any or no reason on [**] prior written notice. Following termination of the Co-Co Agreement as set forth in this subsection (a), Voyager shall not be entitled to any refund or credit for amounts that it may have paid under such Co-Co Agreement prior to termination (other than amounts that may be payable or creditable to Voyager as a final reconciliation of its share of profits and losses through termination).

(b) If Voyager undergoes a Change of Control before the earlier of (i) [**] after the Effective Date or (ii) [**], then Voyager's option rights to participate in the Development and Commercialization under the Co-Co Option shall terminate (unless otherwise agreed by Neurocrine in writing), provided that Voyager's option right to share equally in the Development Costs, Commercialization costs, and profit or loss resulting from the Development and Commercialization of such Products shall not be extinguished and may be exercised in accordance with this Agreement by Voyager or its successor in interest.

(c) If Voyager undergoes a Change of Control during the Term and if the Acquirer is Developing or Commercializing a product that directly competes with a product being Developed or Commercialized by Neurocrine as of the date of the Change of Control, then Neurocrine shall have the right to terminate or amend the Co-Co Agreement (or the Co-Co Option) upon such Change of Control of Voyager, in a manner that will result in Voyager not conducting any Development or Commercialization but the Parties continuing to share (or Voyager or its successor in interest continuing to maintain its option right under the Co-Co Option to share) equally the Development Costs, Commercialization costs and profit or loss resulting from the Development and Commercialization of such Products in the Co-Co Territory.

(d) If the Co-Co Agreement is terminated as set forth in Section 4.1.4(a) or in accordance with the terms of the Co-Co Agreement, then (i) the Co-Co Products from the Co-Co Program shall be deemed Products (and not Co-Co Products) hereunder for the remainder of the Term, (ii) the Parties shall cease to share profit and loss with respect to such Products and Neurocrine's obligation to pay the royalties set forth in Section 8.3.1(a) shall be reinstated from and after the effective date of termination and (iii) Neurocrine's obligations to make milestone payments with respect to such Products shall thereafter be as set forth in Section 8.2(b) for Products that are not Co-Co Products; provided, that Neurocrine shall not have any obligation to make milestone payments with respect to milestones that occurred prior to the effective date of termination of the Co-Co Agreement.

4.2 Neurocrine Development and Commercialization.

4.2.1 Neurocrine Responsibilities. After completion of the Discovery Activities for any Program, Neurocrine shall be solely responsible at Neurocrine's cost and expense for all Development, Manufacturing and Commercialization activities in connection with the Products that are the subject of such Program in the Field in the Territory, which activities shall be conducted in accordance with the Neurocrine Plan and this Agreement; provided that Voyager shall provide reasonable Development assistance to Neurocrine as reasonably requested by Neurocrine and reasonably agreed by Voyager in connection with activities for which Voyager has specific expertise and available current and prospective capacity. Neurocrine shall reimburse Voyager for all Development Costs incurred by Voyager under this Section 4.2.1 in accordance with an agreed plan and budget within [**] of Voyager's submission of an invoice therefor.

4.2.2 Neurocrine Diligence.

(a) Major Market Countries. Neurocrine shall use Commercially Reasonable Efforts: (i) to Develop and seek Regulatory Approval for at least one (1) Product in each Program, which, in the case of the GBA1 Program would include a Product directed to GBA1 Parkinson's disease (or a broader segment of Parkinson's disease) if it would be consistent with the exercise of Commercially Reasonable Efforts to include such Product, in each of [**] (collectively, the "Major Market Countries"); and (ii) to Commercialize at least one Product per Program in each Major Market Country in which it receives Regulatory Approval and, if applicable, Pricing Approval for such Product.

(b) Secondary Market Countries. Neurocrine shall use Commercially Reasonable Efforts: (i) to Develop and seek Regulatory Approval for Products in [**]

(collectively, the "Secondary Market Countries"); and (ii) to Commercialize such Products in the Secondary Market Countries for which it receives Regulatory Approval and, if applicable, Pricing Approval for such Products, to the extent sufficient commercial opportunities exist in such countries and such activities do not impede Development or Commercialization of Products in any Major Market Countries. Notwithstanding the foregoing or any other provision of this Agreement, it may be consistent with the exercise of Commercially Reasonable Efforts for Neurocrine to prioritize one Program over all other Programs and one country over all other countries at any given time.

4.2.3 Neurocrine Plan. Within [**] after completion of the Discovery Activities with respect to a Program, Neurocrine shall submit a written plan, prepared in good faith, (such plan, as each may be amended from time to time in accordance with this Agreement, the "Neurocrine Plan") to the JSC for review and comment, which Neurocrine Plan shall include a description and overall summary of the Development activities that Neurocrine intends to conduct, consistent with Neurocrine's internal practices for the establishment of similar plans and summaries and including, at an appropriate time, the Development activities planned in order to obtain Regulatory Approval for each Product that is the subject of such Program in the Territory, which shall specifically include such activities in each of [**]. Neurocrine shall use Commercially Reasonable Efforts to execute the activities specified in the Neurocrine Plan. Neurocrine shall submit to the JSC material amendments to the Neurocrine Plan from time to time during the term of this Agreement. All material amendments to the Neurocrine Plan shall be reviewed by the JSC.

4.2.4 Neurocrine Reports. Neurocrine shall, at a cadence consistent with the JSC meetings under Section 3.5.2, provide Voyager with written progress reports (which may be through materials prepared for or provided through the JSC) on the status of the Development and Commercialization activities under the applicable Neurocrine Plan with respect to each Product. Notwithstanding the foregoing, Neurocrine agrees that to the extent that an In-License Agreement applicable to a given Program requires more thorough or more frequent reporting or requires that reports be provided on a different timeline than that set forth in this Section 4.2.4, Voyager shall notify Neurocrine of the deadline and content of such reports, sufficiently in advance of the deadline, and provided that Neurocrine has had sufficient notice, Neurocrine shall provide such reports to Voyager as requested by Voyager no less than [**] prior to the date that Voyager is required to submit such report pursuant to the applicable In-License Agreement.

ARTICLE 5
GRANT OF LICENSES

5.1 Licenses to Neurocrine. Subject to the terms and conditions of this Agreement, Voyager hereby grants to Neurocrine, and Neurocrine accepts, an exclusive, royalty-bearing, non-transferable (except in accordance with Section 15.4), sublicenseable (subject to Section 5.4) license, under the Voyager IP, to Exploit Collaboration Candidates and Products (including to conduct research on the Program Capsids therein solely for the purpose of such Exploitation of Collaboration Candidates and Products) in the Field in the Territory during the Term. The foregoing license shall be subject to Voyager's retained rights under the Voyager IP to conduct the activities allocated to Voyager under any Development Plan or Co-Co Agreement or otherwise under this Agreement. The license granted under Section 5.1(b) shall automatically convert to a fully-paid, perpetual, irrevocable royalty-free license on a country-by-country and Product-by-

Product basis upon the expiration of the Royalty Term applicable to such Product in such country (but not upon an earlier termination of this Agreement with respect thereto).

5.2 In-License Agreements.

5.2.1 Scope of Rights under In-License Agreements; Compliance. Neurocrine acknowledges that the license granted by Voyager to Neurocrine in Section 5.1 includes sublicenses under certain Voyager IP that is licensed to Voyager pursuant to In-License Agreements, and that such sublicenses are subject to the applicable terms of the In-License Agreements, the scope of the licenses granted to Voyager or the applicable Affiliate thereunder and the rights granted to or retained by the Third-Party counterparties and any other Third Parties (including Governmental Authorities) (each, an "Inbound Licensor") set forth therein. To the extent Patent Rights under the In-License Agreements are sublicensed to Neurocrine hereunder, Neurocrine covenants to comply with, and to cause its sublicensed Affiliates and to require its Sublicensees to comply with, the In-License Agreements, pursuant to their terms, to the extent provided to Neurocrine prior to the Execution Date or amended in accordance with the terms of this Agreement. To the extent there is a conflict between any of the terms of any In-License Agreement and the rights granted to Neurocrine hereunder (including with respect to any sublicensing rights, Prosecution and Maintenance, enforcement and defense rights) the terms of such In-License Agreement shall control with respect to the Know-How and Patent Rights licensed to Voyager under such In-License Agreement.

5.2.2 Related Third-Party IP.

(a) If either Party becomes aware of any Third-Party's Know-How that would be necessary or reasonably useful for the Exploitation of a Collaboration Candidate or any Third-Party's Patent Right that Covers any Collaboration Candidate in the Territory ("Related Third-Party IP"), such Party shall promptly notify the other Party, and the Parties shall discuss whether to seek a license under such Related Third-Party IP.

(b) Voyager shall have the first right to enter into Third-Party licenses for Related Third-Party IP that Covers a Voyager Capsid with or without a payload (but excluding Related Third-Party IP that specifically Covers a Voyager Capsid with a Program Payload), in Voyager's sole discretion. Notwithstanding the foregoing, if Voyager is negotiating a license under Related Third-Party IP that Covers a Collaboration Candidate, Voyager will provide written notice to Neurocrine and, if Neurocrine expresses a desire to obtain a sublicense under such license pursuant to Section 5.2.3, Voyager shall thereafter (i) provide Neurocrine with a reasonable opportunity to review and comment on the proposed terms of such license that are applicable to Neurocrine as a sublicensee thereunder and (ii) use reasonable efforts to negotiate the terms of such license consistent with Neurocrine's comments, provided that Neurocrine provided its comments within [**] after Neurocrine's receipt of such proposed terms.

(c) Neurocrine shall have the first right to seek any other Third-Party license for any other Related Third-Party IP not subject to Voyager's rights under Section 5.2.2(b). If Neurocrine elects not to seek any such license under other Related Third-Party IP, Neurocrine will provide Voyager written notice of such decision within [**] after the notice described in the

first sentence of Section 5.2.2(a), and if Voyager seeks such license then Neurocrine shall have the rights set forth in Section 5.2.2(b)(i) and (ii) with respect to such Related Third-Party IP.

(d) Notwithstanding anything to the contrary, nothing contained in this Section 5.2.2 creates an obligation for Voyager to obtain any license from a Third-Party.

5.2.3 Future In-Licenses. If, after the Effective Date, subject to Section 5.2.2, Voyager or any of its Affiliates enters into a Future In-License Agreement with a Third-Party pursuant to which Voyager or its Affiliate obtains Control over a Third-Party's Know-How or Patent Rights that would be included within Voyager IP, Voyager shall promptly provide such Future In-License Agreement to Neurocrine and provide any information reasonably requested by Neurocrine with respect thereto, and such Third-Party's Know-How and Patent Rights shall be included in the license granted to Neurocrine under Section 5.1 and considered Voyager IP hereunder, only if Neurocrine agrees in writing to pay the share of the payments due to Inbound Licensors applicable to the Collaboration Candidate(s) or Product(s), as well as a reasonably allocable share of any other payments due to Inbound Licensors not specific to a compound or product, in each case only as and to the extent set forth in Section 5.2.4.

5.2.4 Payments Related to In-License Agreements. As between the Parties, the amounts payable under all In-License Agreements shall be allocated as follows:

(a) With respect to the GBA1 Program (unless and until the GBA1 Program becomes a Co-Co Program) and any New Discovery Program: (i) Voyager shall be responsible for any payment required under applicable Existing In-License Agreements; (ii) Voyager shall be solely responsible for all payments under any Future In-License Agreement for Related Third-Party IP that relates to a Voyager Capsid (and not any other aspect of any Collaboration Candidate or Product), it being agreed that if royalties payable under the Future In-License Agreement exceed the royalties payable by Neurocrine to Voyager with respect to the applicable Collaboration Candidate or Product in the applicable country in the applicable Calendar Quarter, then Neurocrine shall bear such excess; and (iii) for any Future In-License Agreement that is not covered by either clause (i) or clause (ii), Neurocrine will be solely responsible for all payments under any such Future In-License Agreement for Related Third-Party IP that relates to any component of a Collaboration Candidate or Product other than a Voyager Capsid. Notwithstanding the foregoing, Voyager shall be solely responsible for all payments under any potential In-License Agreements for intellectual property referenced on Schedule 5.2.4(a) for the GBA1 Program.

(b) With respect to the Co-Co Program, from and after the exercise of the Co-Co Option, pursuant to Section 4.1.2(d), any amounts paid to any Inbound Licensor under an In-License Agreement (including royalty, milestone and sublicense income payments) with respect to the Co-Co Products shall be shared between the Parties as Co-Co Product costs, to the extent such amounts are allocable to the Co-Co Product in the Co-Co Territory, in accordance with Sections 4.1.2 and 4.1.3.

5.2.5 Neurocrine shall prepare and deliver to Voyager any additional reports required under the applicable In-License Agreements of Voyager, in each case to the extent requested by Voyager, and, provided that Voyager has notified Neurocrine reasonably sufficiently

in advance of the applicable deadline, to enable Voyager to comply with its obligations under the applicable In-License Agreements.

5.3 Obligations Under In-Licenses.

5.3.1 Voyager shall not take (or fail to take) any action, including failure to pay any amounts when due (except that any such failure to pay that was caused by Neurocrine's failure to make a payment required to be made by Neurocrine under Section 5.2.4 will not be considered an action or failure to take action by Voyager for purposes of this Section 5.3.1), that constitutes a material breach under any In-License Agreement. Voyager will not, without the consent of Neurocrine: (a) take any action with respect to any In-License Agreement (including amending, terminating or otherwise modifying) that diminishes the rights granted to Neurocrine under this Agreement; or (b) fail to take any action with respect to an In-License Agreement that is reasonably necessary to avoid diminishing the rights granted to Neurocrine under this Agreement.

5.3.2 Voyager shall reasonably enforce, or otherwise take all actions necessary to enable Neurocrine to enforce, at Voyager's expense, Voyager's rights and benefits and the obligations of the counterparty under each In-License Agreement that may affect the rights, benefits and obligations of Neurocrine hereunder, including taking such actions as Neurocrine may request, and will inform Neurocrine of any action it takes under any In-License Agreement to the extent such action may affect Neurocrine's rights under this Agreement.

5.3.3 Voyager shall not (and shall cause its Affiliates not to) assign (except an assignment to a party to which this Agreement has been assigned as permitted under Section 15.4) any In-License Agreement without the prior written consent of Neurocrine.

5.3.4 Voyager shall (and shall cause its Affiliates to) provide Neurocrine with prompt notice of any claim of a breach under any In-License Agreement or notice of termination of any In-License Agreement, made by any of Voyager, its Affiliate or the Inbound Licensor, and shall promptly send to Neurocrine (or cause its Affiliates promptly to send to Neurocrine) copies of all material correspondence regarding each In-License Agreement, to the extent relevant to the rights or obligations of Neurocrine under this Agreement.

5.3.5 In the event that Voyager or its Affiliate receives written notice of an alleged breach by Voyager or its Affiliate under any In-License Agreement, where termination of such In-License Agreement or any diminishment of the licenses granted to Neurocrine under the Voyager IP is being or could be sought by the Inbound Licensor, then Voyager will promptly, but in no event less than [**] thereafter, provide written notice thereof to Neurocrine and grant Neurocrine the right (but not the obligation) to cure such alleged breach, and if Neurocrine elects to and does cure such breach, then Neurocrine may offset any Out-of-Pocket Costs and expenses incurred by or on behalf of Neurocrine or any of its Affiliates in connection with curing such breach against Neurocrine's future payment obligations to Voyager under this Agreement. Each Party shall notify the other Party if it intends to cure such breach and again promptly after curing such breach.

5.3.6 Neurocrine acknowledges and agrees that, if any license granted to Voyager under an In-License Agreement is terminated then Neurocrine's sublicense under such terminated license shall automatically terminate, subject to Neurocrine's right to receive a direct license from

any Inbound Licensor of such In-License Agreement to the extent specified in the applicable In-License Agreement. In the event that any In-License Agreement is terminated by the applicable Inbound Licensor, and such In-License Agreement does not permit the sublicense to survive (or Neurocrine to receive a direct license), then Voyager will take all reasonable actions requested by Neurocrine to facilitate Neurocrine's entry into a direct license agreement with the applicable Inbound Licensor. In the event that any In-License Agreement is terminated by the applicable Inbound Licensor, and such In-License Agreement permits the sublicense to survive (or Neurocrine to receive a direct license), Neurocrine will have the right, at Neurocrine's election, to convert the applicable sublicenses granted under this Agreement by Voyager to a direct license from the applicable Inbound Licensor to Neurocrine on the terms and conditions contained in such In-License Agreement, or such other terms and conditions as may be negotiated by Neurocrine and the applicable Inbound Licensor, and Voyager will reasonably cooperate with Neurocrine and its Affiliates to effectuate such direct license and assist Neurocrine in discussions with Inbound Licensors to accomplish such direct license. In the event Neurocrine enters into any such direct license with an Inbound Licensor, Neurocrine may offset any Out-of-Pocket Costs and expenses incurred by or on behalf of Neurocrine or any of its Affiliates or Sublicensees in connection with entering into and exercising its rights or performing under such direct license, against Neurocrine's future payment obligations to Voyager under this Agreement.

5.4 Neurocrine's Sublicensing Rights. Neurocrine shall have the right to grant and authorize sublicenses under the rights granted to it under Section 15.1 to any of its Affiliates and Third Parties through multiple tiers (each such Third-Party, a "Sublicensee"). Neurocrine shall provide Voyager with a fully executed copy of any agreement (redacted as necessary to protect confidential or commercially sensitive information that is not necessary for Voyager to determine Neurocrine's compliance with this Agreement or for Voyager to comply with any applicable In-License Agreement) reflecting any such sublicense to a Third-Party promptly after the execution thereof (a "Sublicense"). If Neurocrine or any Affiliate or Sublicensee grants a sublicense, the terms and conditions of this Agreement that are applicable to Sublicensees shall apply to such Sublicensee to the same extent as they apply to Neurocrine. Neurocrine will itself pay and account to Voyager for all payments due under this Agreement by reason of operation of any such sublicense. Each Sublicense must be consistent with, and require the Sublicensee to meet, all applicable obligations and requirements of the In-License Agreements. Notwithstanding the foregoing, unless and until the receipt of written agreement by the applicable Inbound Licensor to permit further sublicensing to a Third-Party, Neurocrine shall not have the right to grant any sublicenses to the extent not permitted under the applicable In-License Agreement; provided that upon Neurocrine's request, Voyager will use reasonable, good faith efforts to obtain the right for Neurocrine to grant sublicenses to the extent not already permitted by an In-License Agreement.

5.5 Licenses to Voyager.

5.5.1 Development License. Subject to the terms and conditions of this Agreement, Neurocrine hereby grants to Voyager, and Voyager accepts, a non-exclusive, royalty-free, non-transferable (except in accordance with Section 15.4), sublicenseable (only to its permitted subcontractors under Section 7.6) license under the Neurocrine IP solely to conduct the Development and Manufacturing activities allocated to Voyager under the Development Plans in the Field in the Territory in accordance with this Agreement.

5.5.2 Co-Co License. Subject to the terms and conditions of this Agreement and the Co-Co Agreement, upon Voyager's exercise of the Co-Co Option in accordance with Section 4.1.1, Neurocrine grants to Voyager, and Voyager accepts, a non-exclusive, non-transferable (except in accordance with Section 15.4), sublicenseable (solely as set forth in the Co-Co Agreement) license under the Neurocrine IP to conduct those Exploitation activities that are allocated to Voyager under the Co-Co Agreement with respect to Co-Co Products in the Co-Co Program in the Field in and for the Co-Co Territory during the term of the Co-Co Agreement.

5.6 No Other Rights. Except as otherwise expressly provided in this Agreement, under no circumstances shall a Party, as a result of this Agreement, obtain any ownership interest, license right or other right in any Know-How, Patent Rights or other intellectual property rights of the other Party or any of its Affiliates, including items owned, controlled, developed or acquired by the other Party or any of its Affiliates, or provided by the other Party to the first Party at any time pursuant to this Agreement.

5.7 Section 365(n) of the Bankruptcy Code. All rights and licenses granted under or pursuant to this Agreement by a Party to the other, including those set forth in Sections 5.1 and 5.6, are and shall otherwise be deemed to be, for purposes of Section 365(n) of Title 11 of the U.S. Bankruptcy Code ("Title 11"), licenses of rights to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The Parties agree that the Parties shall retain and may fully exercise all of their rights and elections under the U.S. Bankruptcy Code and any foreign counterpart thereto. Without limiting the Parties' rights under Section 365(n) of Title 11, if a case under Title 11 is commenced by or against either Party, the other Party shall be entitled to a copy of any and all such intellectual property and all embodiments of such intellectual property, and the same, if not in the possession of such other Party, shall be promptly delivered to it: (a) before this Agreement is rejected by or on behalf of such Party, within [**] after such other Party's written request, unless such Party, or its trustee or receiver, elects within [**] to continue to perform all of its obligations under this Agreement; or (b) after any rejection of this Agreement by or on behalf of such Party, if not previously delivered as provided under clause (a) above (any such event described in clause (a) or (b) above, and occurring while such Title 11 case is pending, being a "Delivery Event"). All rights of the Parties under this Section 5.7 and under Section 365(n) of Title 11 are in addition to and not in substitution of any and all other rights, powers, and remedies that each Party may have under this Agreement, Title 11, and any other applicable Laws. The Parties agree that they intend the foregoing rights to extend to the maximum extent permitted by Law and any provisions of applicable contracts with Third Parties, including for purposes of Title 11: (x) the right of access to any intellectual property (including all embodiments thereof) of Voyager or Neurocrine, as applicable, or any Third-Party with whom Voyager or Neurocrine contracts to perform an obligation of Voyager or Neurocrine under this Agreement, and, in the case of the Third-Party, that is necessary for the Development and Manufacture of Collaboration Candidates or Products; and (y) the right to contract directly with any Third-Party described in clause (x) in this sentence to complete the contracted work, provided however, that in each case such rights shall be subject to the confidentiality obligations contemplated by this Agreement. If a bankruptcy proceeding is commenced by or against Voyager, notwithstanding anything to the contrary in ARTICLE 10, Neurocrine may, to the maximum extent permitted by Law, take appropriate actions in connection with the filing, prosecution, maintenance and enforcement of any Voyager Patent Rights licensed to Neurocrine under this Agreement to the extent that Voyager is required or has

the right to take such actions under this Agreement and to the extent that Voyager fails to take such actions following at least [**] prior written notice from Neurocrine.

ARTICLE 6
MANUFACTURING

6.1 Manufacturing Responsibilities During the Discovery Period. The Development Plans shall specify the allocation between the Parties of responsibilities for the Manufacture of Collaboration Candidates associated with the applicable Program during the Discovery Period, and, if Voyager conducts any portion of the Manufacture of such Collaboration Candidates, the Development Plan(s) shall also include an obligation for Voyager to assist with the technology transfer of such Manufacturing responsibilities to Neurocrine or a Third-Party contract manufacturing organization, as reasonably requested by Neurocrine, on terms to be mutually agreed by the Parties.

6.2 Manufacturing Responsibilities After the Discovery Period. Unless otherwise agreed by the Parties in writing, Neurocrine shall be responsible for the Manufacture of all Collaboration Candidates and Products after the end of the Discovery Period.

ARTICLE 7
GENERAL PROVISIONS RELATING TO ACTIVITIES

7.1 Compliance. All Development, Manufacturing and Commercialization activities to be conducted by a Party under this Agreement shall be conducted in compliance with applicable Laws, including all applicable cGMP, GLP and GCP requirements.

7.2 Regulatory Activities.

7.2.1 INDs and Related Communications.

(a) Subject to the terms of any applicable Co-Co Agreement, from and after the end of the Discovery Period, Neurocrine shall, as between the Parties, have the sole right to prepare, obtain and maintain all INDs, Regulatory Approval Applications (including the setting of the overall regulatory strategy therefor), other Regulatory Approvals, pricing and reimbursement approvals and other submissions and to conduct communications with the Regulatory Authorities and Governmental Authorities in the Territory for the applicable Products. Neurocrine will be the regulatory sponsor for all Clinical Trials commenced on Products from and after the Effective Date. Voyager's rights with respect to regulatory interactions for the Co-Co Product shall be as set forth in the Co-Co Agreement.

(b) Prior to [**] following the Co-Co Trigger Date (and if Voyager exercises the Co-Co Option, thereafter until entry into the Co-Co Agreement and thereafter in accordance with such Co-Co Agreement), or if earlier, Voyager's notification to Neurocrine that it will not exercise the Co-Co Option, Neurocrine shall provide Voyager with drafts of each Regulatory Approval Application or other material submission or communication described in Section 7.2.1(a) with respect to any Product in the GBA1 Program for Voyager's review and comment a reasonable period of time prior to submission thereof. At Neurocrine's discretion (with

Neurocrine having final decision-making authority), Neurocrine shall, and shall cause its Affiliates to, reasonably incorporate any comments of Voyager into such Regulatory Approval Applications and other material submissions and communications if received by Neurocrine within a reasonable period of time after Neurocrine has provided access to Voyager.

(c) Prior to [**] following the Co-Co Trigger Date (and if Voyager exercises the Co-Co Option, thereafter until entry into the Co-Co Agreement and thereafter in accordance with such Co-Co Agreement), or if earlier, Voyager's notification to Neurocrine that it will not exercise the Co-Co Option, Neurocrine shall provide Voyager with prior written notice, to the extent Neurocrine has advance knowledge, of any scheduled meeting, conference, or discussion (including any advisory committee meeting) with a Regulatory Authority in the Territory relating to any substantive matter with respect to any Product in the GBA1 Program, within [**] after Neurocrine or its Affiliate first receives notice of the scheduling of such meeting, conference, or discussion (or within such shorter period as may be necessary in order to give Voyager a reasonable opportunity to attend such meeting, conference, or discussion). If permitted by Neurocrine in its reasonable discretion, Voyager shall have the right to have one (1) or, to the extent reasonable, more of its Representatives attend (and, if permitted by Neurocrine and the applicable Regulatory Authority, participate) in all such meetings, conferences, and discussions.

(d) Notwithstanding anything to the contrary, this Section 7.2.1 shall not in any way prohibit Neurocrine from complying with its reporting requirements pursuant to applicable Law, including with respect to adverse event reporting.

7.2.2 Ownership and Assignment of Regulatory Filings. All Regulatory Filings (including all Regulatory Approvals) and pricing and reimbursement approvals in the Territory with respect to the applicable Products shall be owned by, and shall be the sole property and held in the name of, Neurocrine or its designated Affiliate, Sublicensee or designee.

7.2.3 Right of Reference. Voyager hereby grants to Neurocrine a "Right of Reference," as that term is defined in 21 C.F.R. 314.3(b) (or any analogous Law recognized outside of the United States), to all data Controlled by Voyager or any of its Affiliates that relate to any Program Capsid, Collaboration Candidate or Product solely for purposes of seeking Regulatory Approval for Products, and Voyager shall provide a signed statement to this effect, if requested by Neurocrine, in accordance with 21 C.F.R. 314.50(g)(3) (or any analogous Law outside of the United States).

7.3 Sale of Priority Review Voucher. If the FDA grants to Neurocrine a priority review voucher in connection with the approval of the BLA for a Product (a "PRV"), Neurocrine may: (a) sell the PRV to a Third-Party in an arm's-length transaction (a "PRV Sale"); (b) keep the PRV for its own use or use by any of its Affiliates for any product other than a Product (a "Neurocrine PRV Use"); or (c) use the PRV for a Product.

7.3.1 In the event of a PRV Sale: (a) if the PRV was for a Product in the GBA1 Program and the Co-Co Option was either previously exercised or had not expired or been waived by Voyager, then Neurocrine shall pay Voyager an amount equal to [**]; and (b) with respect to the PRV for any Product from the GBA1 Program after expiration of the Co-Co Option without exercise or any Product from any New Discovery Program, Neurocrine shall pay Voyager an

amount equal to the [**]. In the event the Co-Co Option has been exercised as of the time of a PRV Sale and the Reimbursable Costs associated with exercise of the Co-Co Option have not already been paid by Voyager, if the Reimbursable Costs exceed [**], then the [**] shall constitute a credit against the Reimbursable Costs, and the Reimbursable Costs will be reduced by an equal measure.

7.3.2 In the Event of a Neurocrine PRV Use: (a) if (i) the PRV was for a Product in the GBA1 Program and the Co-Co-Option was either previously exercised or had not expired or been waived by Voyager, then (ii) Neurocrine shall pay Voyager an amount equal to [**]; and (b) with respect to the PRV for any Product from the GBA1 Program after expiration of the Co-Co Option without exercise or any Product from any New Discovery Program, Neurocrine shall pay Voyager an amount equal to the [**]. All payments under this Section 7.3 shall be made within [**] after the closing of the PRV Sale or the effective date of Neurocrine PRV Use, as applicable.

7.3.3 If Neurocrine uses the PRV for a Product, no payments will be due to Voyager under this Section 7.3.

7.4 Records and Audits. Each Party shall, and shall require its Affiliates and permitted subcontractors to, maintain complete, current and accurate hard and electronic (as applicable) copies of records of all work conducted pursuant to its Development, Manufacturing and Commercialization activities under this Agreement, and all results, data, developments and Know-How made in conducting such activities. Such records shall fully and accurately reflect all such work done and results achieved in sufficient detail and in good scientific manner appropriate for applicable patent and regulatory purposes. Each Party shall document all non-clinical studies and clinical trials for Programs in formal written study records according to applicable Laws, including national and international guidelines such as the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, GCP, GLP and cGMP. Neurocrine shall have the right to receive and retain a copy of all such records of Voyager at reasonable times, upon reasonable prior written notice to Voyager, during and after the end of the Discovery Period with regard to all such records relating to the Development or Manufacturing activities conducted by Voyager hereunder. Neurocrine agrees that to the extent that an In-License Agreement applicable to a given Program requires records to be retained for a period longer than the period set forth in this Section 7.4, Neurocrine shall retain applicable records for such time period as required by the applicable In-License Agreement.

7.5 No Representation. No Party makes any representation, warranty or guarantee that the Collaboration will be successful, or that any other particular results will be achieved with respect to the Collaboration, any Program, any Collaboration Candidate or Product.

7.6 Subcontracting. Subject to the terms of this Agreement, each Party shall have the right to engage Affiliates or Third-Party subcontractors (including contract manufacturing organizations) to perform its Development or Manufacturing obligations under this Agreement. Any such Affiliate or subcontractor shall meet the qualifications typically required by such Party for the performance of work similar in scope and complexity to the subcontracted activity and perform such work consistent with the terms of this Agreement; provided, however, that a Party engaging an Affiliate or subcontractor hereunder shall remain fully responsible and obligated for all activities performed by such Affiliate or subcontractor. Unless otherwise agreed by the Parties,

each Party will obligate each of its Third-Party subcontractors hereunder to agree in writing to assign to such Party ownership of, or, solely after using reasonable efforts to obtain such an assignment and being unable to obtain such an assignment, grant to such Party an exclusive, royalty-free, worldwide, perpetual and irrevocable license (with the right to freely grant sublicenses through multiple tiers) to, any Inventions arising under its agreement with such Third-Party to the extent related to or resulting from the Development, Manufacture or Commercialization of Collaboration Candidates or Products; and such Party shall structure such assignment or exclusive license so as to enable such Party to license or sublicense such Third-Party Inventions to the other Party pursuant to the applicable provisions of this Agreement (including permitting such other Party to grant further sublicenses in accordance with this Agreement).

7.7 Academic Collaborators. If any Party collaborates with an academic institution or one or more individuals at an academic institution to Develop Collaboration Candidates or Products, such Party shall be required to use reasonable efforts to obligate such academic collaborator to agree in writing to grant the same rights specified in Section 7.6 with respect to ownership or licenses to Inventions; it being understood and agreed that, in lieu of the rights specified in Section 7.6, it shall be sufficient for such Party to obtain a non-exclusive, worldwide, royalty-free, perpetual license (with the right to freely grant sublicenses through multiple tiers) to, and a right to negotiate for an exclusive license, with the right to grant sublicenses to, any such Inventions, which sublicensing rights must permit sublicensing to the other Party pursuant to the applicable provisions of this Agreement (including permitting such other Party to grant further sublicenses in accordance with this Agreement); provided that if such Party is unable to obtain such non-exclusive license and right to negotiate for an exclusive license, despite the use of commercially reasonable efforts, then (a) in the case of academic collaborations that are not reasonably expected by the applicable Party to result in Inventions related to the composition of matter of any Capsid, (i) for the Co-Co Program, the Parties shall determine, and for all other Programs, Neurocrine shall determine whether it is sufficient to obtain the broadest rights reasonably possible, with respect to ownership or licenses to Inventions, as is commercially reasonable and customary with the applicable institution, and (b) in the case of academic collaborations that are reasonably expected by the applicable Party to result in Inventions related to the composition of matter of any Capsid, the terms with respect to ownership or licenses to Inventions shall be subject to Voyager's approval.

ARTICLE 8
INITIAL FEE; MILESTONES AND ROYALTIES; PAYMENTS

8.1 Initial Consideration.

8.1.1 Upfront Fee. In partial consideration for the rights granted to Neurocrine hereunder, Neurocrine shall pay Voyager a one-time, non-refundable, non-creditable upfront cash payment equal to the difference between One Hundred Seventy-Five Million Dollars ($175,000,000) and the amount paid by Neurocrine pursuant to the Stock Purchase Agreement within five (5) Business Days after the Effective Date. Such upfront cash payment shall be allocated as set forth on Schedule 8.1.1.

8.1.2 Equity Purchase. In partial consideration of the rights granted hereunder, on the Effective Date, Voyager shall issue and sell to Neurocrine, and Neurocrine shall purchase from Voyager, that number of shares of Common Stock that, when combined with the shares of Common Stock owned by Neurocrine immediately prior to the Effective Date, results in Neurocrine owning 19.9% of Voyager's outstanding Common Stock immediately following the Effective Date, pursuant to the terms of the stock purchase agreement attached as Exhibit A (the "Stock Purchase Agreement") and executed by the Parties concurrently with this Agreement.

8.2 Milestone Payments.

(a) Each event described in Sections 8.2.1, 8.2.2 and 8.2.3 is referred to as a "Milestone Event." In partial consideration for the rights and licenses granted to Neurocrine hereunder: (i) within [**] after (A) in the case of Milestone Event (a) under Section 8.2.1 and Milestone Event (a) under Section 8.2.2, the JSC's determination that such Milestone Event was achieved pursuant to Section 2.1.6 (or, as applicable, the date upon which Neurocrine or its Affiliate or Sublicensee [**]), and (B) in all other cases under Sections 8.2.1 and 8.2.2, the first achievement of a Milestone Event set forth below by or on behalf of Neurocrine, any of its Affiliates or any Sublicensee; and (ii) in the case of Section 8.2.3, within [**] after the end of the Calendar Quarter in which achievement of the applicable Commercial Milestone first occurs, in each case (i) and (ii), Neurocrine shall make a one-time (except as provided below), non-refundable, non-creditable milestone payment to Voyager in the amount below corresponding to such Milestone Event (each, a "Milestone Payment").

(b) If Voyager does not timely exercise its Co-Co Option with respect to the GBA1 Program, then the tables in Section 8.2.1 (for Development Milestones) and Section 8.2.3 (for Commercial Milestones) shall apply in their entirety with respect to the GBA1 Program. If Voyager exercises its Co-Co Option with respect to the GBA1 Program, then Voyager shall be entitled to receive Milestone Payments only with respect to any Milestone Event that relates to the Territory outside the Co-Co Territory for so long as the GBA1 Program remains a Co-Co Program, as further provided below. If the Co-Co Agreement is terminated and the GBA1 Program is no longer a Co-Co Program, then the tables in Section 8.2.1 (for Development Milestones), and Section 8.2.3 (for Commercial Milestones) shall thereafter apply with respect to the GBA1 Program in the United States, but only with respect to Milestone Events achieved after termination of the Co-Co Agreement.

(c) Except as expressly set forth below, each Milestone Payment shall be deemed earned as of the achievement of the corresponding Milestone Event.

8.2.1 Development Milestone Payments for Development Candidates and Products under the GBA1 Program.

	Milestone Event	Milestone Payment ($)
(a)	[**]	[**]

	Milestone Event	Milestone Payment ($)	
		First Indication	Second Indication
(b)	[**]	[**]	[**]
(c)	[**]	[**]	[**]
(d)	[**]	[**]	[**]
(e)	[**]	[**]	[**]
(f)	[**]	[**]	[**]
(g)	[**]	[**]	[**]
(h)	[**]	[**]	[**]
(i)	[**]	[**]	[**]

The Milestone Payment described in Section 8.2.1(a) may be paid for each of [**]. All other Milestone Payments above may be paid for up to two (2) Products for the GBA1 Program.

In the event the Development Milestone described in Section 8.2.1(b) is not required for a Product Developed [**], the Development Milestone described in Section 8.2.1(b) shall be due and payable. In the event the Development Milestone described in Section 8.2.1(d) occurs with respect to a Product and Indication but the Milestone Event described in Section 8.2.1(c) has not occurred and the corresponding Milestone Payment has not been paid for such Product and Indication, then the Milestone Payment associated with the Milestone Event described in Section 8.2.1(c) shall be due and payable with the payment associated with the Development Milestone described in Section 8.2.1(d). In the event the Development Milestone described in Section 8.2.1(f) occurs with respect to a Product and Indication but any of the Milestone Events described in Section 8.2.1(c), 8.2.1(d) or 8.2.1(e) has not occurred and the corresponding Milestone Payment has not been paid for such Product and Indication, then the Milestone Payment associated with each of the Milestone Events described in Section 8.2.1(c), 8.2.1(d) or 8.2.1(e) that were previously unpaid, as applicable, shall be due and payable with the payment associated with the Development Milestone described in Section 8.2.1(f). In the event the Development Milestone described in Section 8.2.1(g) occurs with respect to a Product and Indication, any prior such Development

Milestones that have not occurred for such Product and Indication shall be deemed to have occurred, and any Milestone Payment(s) associated with such prior Development Milestones that have not previously been paid shall be due and payable with the Milestone Payment associated with the Milestone Event described in Section 8.2.1(g).

8.2.2 Development Milestone Payments for Development Candidates and Products under New Discovery Programs.

	Milestone Event	**Milestone Payment ($)**
(a)	[**]	[**]
(b)	[**]	[**]
(c)	[**]	[**]
(d)	[**]	[**]
(e)	[**]	[**]
(f)	[**]	[**]
(g)	[**]	[**]
(h)	[**]	[**]

The Milestone Payment described in Section 8.2.2(a) may be paid for [**]. All other Milestone Payments above may be paid only one (1) time per New Discovery Program.

In the event the Development Milestone described in Section 8.2.2(d) occurs with respect to a New Discovery Program but the Milestone Event described in Section 8.2.2(c) has not occurred and the corresponding Milestone Payment has not been paid for such New Discovery Program, then the Milestone Payment associated with the Milestone Event described in Section 8.2.2(c) shall be due and payable with the payment associated with the Development Milestone described in Section 8.2.2(d). In the event the Development Milestone described in Section 8.2.2(e) occurs with respect to a New Discovery Program but any of the Milestone Event described in Section 8.2.2(c) or 8.2.2(d) has not occurred and the corresponding Milestone Payment has not been paid for such New Discovery Program, then the Milestone Payment associated with each of the Milestone Events described in Section 8.2.2(c) or 8.2.2(d) that were previously unpaid, as applicable, shall be due and payable with the payment associated with the Development Milestone described in Section 8.2.2(e). In the event the Development Milestone described in Section 8.2.2(f) occurs with respect to a New Discovery Program, any prior such Development Milestones that have not occurred for such New Discovery Program shall be deemed to have occurred, and any Milestone Payment(s) associated with such prior Development Milestones that have not previously been paid shall be due and payable with the Milestone Payment associated with the Milestone Event described in Section 8.2.2(f).

8.2.3 Commercial Milestones for Products under the GBA1 Program.

	Milestone Event	$ in Millions
(a)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]
(b)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]
(c)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]
(d)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]
(e)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]
(f)	Annual Territory-wide (except as provided below) Net Sales of such Product greater than or equal to $[**]	[**]

The Milestone Payments above will be payable up to two (2) times for each of up to two (2) Products in the GBA1 Program to achieve the corresponding Milestone Event; provided, however that no Product that is approved solely for [**] shall result in Milestone Payments and count against the up to two (2) Products for which Voyager is eligible for Milestone Payments under this Section 8.2.3. To the extent any Product is approved only, or initially only, for [**], sales of such Product for [**] will not count toward Milestone Payments under this Section 8.2.3 unless and until such Product is also approved for [**] and is otherwise eligible to support Milestone Payments under this Section 8.2.3. With respect to Co-Co Products, Net Sales in the Co-Co Territory will not be included in aggregate Net Sales for purposes of determining whether the Commercial Milestones above have been achieved.

8.2.4 Commercial Milestones for Products under the New Discovery Programs.

	Milestone Event	$ in Millions
(a)	Annual Territory-wide Net Sales of such Product greater than or equal to $[**]	[**]
(b)	Annual Territory-wide Net Sales of such Product greater than or equal to $[**]	[**]
(c)	Annual Territory-wide Net Sales of such Product greater than or equal to $[**]	[**]
(d)	Annual Territory-wide Net Sales of such Product greater than or equal to $[**]	[**]

The Milestone Payments above will be payable one time for each New Discovery Program, as measured in aggregate across all Products, to achieve the corresponding Milestone Event.

8.3 Royalties. Subject to the adjustments under Section 8.5, Neurocrine will make royalty payments, during the applicable Royalty Terms, as set forth in this Section 8.3.

8.3.1 Royalties on Products under GBA1 Program.

(a) Annual Net Sales in the United States. In further consideration for the licenses and other rights granted to Neurocrine with respect to the GBA1 Program, Neurocrine shall make tiered royalty payments to Voyager in respect of Annual Net Sales in the United States, on a Product-by-Product basis, of Products under the GBA1 Program that are not Co-Co Products.

	Annual Net Sales in the United States of the Product	**Tiered Royalty Rate**
(a)	Annual Net Sales in the United States less than [**] Dollars ($[**])	[**] Percent ([**]%)
(b)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(c)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(d)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**])	Twenty Percent (20%)

(b) Annual Net Sales outside of the United States. In further consideration for the licenses and other rights granted to Neurocrine with respect to the GBA1 Program, Neurocrine shall make tiered royalty payments to Voyager in respect of Annual Net Sales in the Territory outside the United States, on a Product-by-Product basis, of Products under the GBA1 Program.

	Annual Net Sales outside the United States of the Product	**Tiered Royalty Rate**
(a)	Annual Net Sales outside the United States less than [**] Dollars ($[**])	[**] Percent ([**]%)
(b)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(c)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(d)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($[**])	[**] Percent ([**]%)

8.3.2 Product Royalties on Products under New Discovery Programs

(a) Annual Net Sales in the United States. In further consideration of the licenses and other rights granted to Neurocrine with respect to each New Discovery Program, Neurocrine shall make tiered royalty payments to Voyager in respect of Annual Net Sales in the United States, on a Product-by-Product basis, of Products under each New Discovery Program.

	Annual Net Sales in the United States of the Product	Tiered Royalty Rate
(a)	Annual Net Sales in the United States less than [**] Dollars ($[**])	[**] Percent ([**]%)
(b)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(c)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(d)	Annual Net Sales in the United States greater than or equal to [**] Dollars ($[**])	[**] Percent ([**]%)

(b) Annual Net Sales outside of the United States. In further consideration of the licenses and other rights granted to Neurocrine with respect to each New Discovery Program, Neurocrine shall make tiered royalty payments to Voyager in respect of Annual Net Sales in the Territory outside the United States, on a Product-by-Product basis, of Products under each New Discovery Program.

	Annual Net Sales outside the United States of the Product	Tiered Royalty Rate
(a)	Annual Net Sales outside the United States less than [**] Dollars ($[**])	[**] Percent ([**]%)
(b)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(c)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($1[**]) but less than [**] Dollars ($[**])	[**] Percent ([**]%)
(d)	Annual Net Sales outside the United States greater than or equal to [**] Dollars ($[**])	[**] Percent ([**]%)

8.3.3 Calculation of Royalties. Royalties on aggregate Net Sales of Products in a Calendar Year shall be paid at the rate applicable to the portion of Net Sales within each of the Annual Net Sales tiers during such Calendar Year. For example, if, during a Calendar Year, Annual Net Sales of Products under the GBA1 Program in the United States are equal to $[**], then the royalties payable by Neurocrine would equal $[**], calculated by adding: [**].

8.4 Royalty Period. On a country-by-country and Product-by-Product basis, royalty payments in the Territory shall commence on the First Commercial Sale of such Product in such country and terminate upon the latest of: (a) the expiration, invalidation or abandonment date of the last Valid Claim of the Voyager Patent Rights or Joint Patent Rights that claims the composition of matter or method of use (for an Indication for which such Product received Regulatory Approval in such country) of such Product in such country; (b) ten (10) years from

First Commercial Sale of such Product in such country; and (c) expiration of Regulatory Exclusivity for such Product in such country (the applicable "Royalty Term").

8.5 Royalty Adjustments.

8.5.1 Valid Claim Expiration. If, with respect to a Product in any country in the Territory, at any time in the Royalty Term for such Product and country there is no Valid Claim within the Voyager Patent Rights or the Joint Patent Rights that claims the composition of matter or method of use (for an indication for which such Product received Regulatory Approval in such country) of such Product in such country, then the royalties payable for such Product in such country shall be reduced by fifty percent (50%) from the royalties otherwise due for such Product in such country under Section 8.3. If such royalty reduction applies to any country other than the United States, it will be calculated by determining the portion of total Net Sales in the Territory outside the United States of the relevant Product in a Calendar Quarter that is attributable to the country in which such reduction applies, and by determining the total royalties for the Territory outside the United States without reduction, and then reducing by fifty percent (50%) the applicable portion (based on Net Sales) of the total royalties attributable to the country in which such reduction applies.

8.5.2 Biosimilar Reduction. If, in any country in the Territory during the Royalty Term in such country for a Product, a Biosimilar Product with respect to such Product is launched in such country, then, for any Calendar Quarter in which such Biosimilar Product(s) comprise greater than or equal to [**] percent ([**]%) of the total units of such Product and Biosimilar Product(s) sold in such country (based on sales of units of such Product and Biosimilar Product(s) as reported by IQVIA, or, if such data are not available, such other reliable data source as reasonably determined by Voyager and Neurocrine) the royalties payable for such Product with respect to such country for such Calendar Quarter shall be reduced by fifty percent (50%) from the royalties otherwise due for such Product in such country under Section 8.3. Such reduction shall be calculated as described in the last sentence of Section 8.5.1.

8.5.3 Stacking. If (a) Neurocrine or any of its Affiliates determines in good faith that it is reasonably necessary to (i) obtain a license from a Third-Party under one or more Valid Claims licensable by such Third-Party Covering a Product or under Know-How licensable by such Third-Party in order for Neurocrine, its Affiliates and Sublicensees to Exploit such Product in the Field in a country in the Territory and (ii) make payments under such license, and Neurocrine or any of its Affiliates actually enters into any such license, or makes payments to Voyager under Section 5.2.4(a), then (b) the amount of Neurocrine's royalty payments under Section 8.3 for such Product in such country in a Calendar Quarter may be reduced by fifty percent (50%) of the royalties and other amounts actually paid by Neurocrine or any of its Affiliates to Voyager or such Third-Party to the extent applicable to such Product in such country during such Calendar Quarter; provided, however, that neither Neurocrine nor any of its Affiliates shall be entitled to make reductions hereunder for any amounts payable by Neurocrine or any of its Affiliates relating to any Neurocrine IP existing as of the Effective Date. In addition to the foregoing, Neurocrine may credit [**], against Neurocrine's royalty payments under Section 8.3 for such Product and such credit may be carried forward to subsequent Calendar Quarters until the total of such amounts with respect to such Product has been fully credited against royalty payments.

8.5.4 Limits on Deductions. On a Product-by-Product basis, in no event shall the cumulative effect of the adjustments in Sections 8.5.1, 8.5.2 or 8.5.3 reduce the royalties payable to Voyager pursuant to Section 8.3 below fifty percent (50%) of the amounts that would otherwise have been payable with respect to the applicable Product in the applicable country in the applicable Calendar Quarter, as determined pursuant to Section 8.3.3. Neurocrine may carry forward to subsequent Calendar Quarters any amounts it could not deduct as a result of the application of the preceding sentence.

8.6 Reports; Payment of Royalty.

8.6.1 Reports. During the Term, following the First Commercial Sale of any Product in any country in the Territory (excluding the First Commercial Sale in the United States of a Co-Co Product for which reporting shall be addressed in the Co-Co Agreement), Neurocrine shall furnish to Voyager a written report within [**] after the end of each Calendar Quarter showing, on a Product-by-Product and country-by-country basis, the Net Sales of each Product in each country of the Territory and the royalties payable under this Agreement. Royalties with respect to Net Sales of Products shall be due and payable on the date such royalty report is due to Voyager.

8.6.2 Compliance with In-License Agreements. Neurocrine and its Affiliates and Sublicensees shall provide any information reasonably requested by Voyager to enable Voyager to comply with any applicable reporting requirements under the In-License Agreements. Provided that Voyager timely notifies Neurocrine of such reporting requirement, Neurocrine shall ensure that all applicable and necessary information is received by Voyager from Neurocrine, whether generated by Neurocrine, any of its Affiliates or any Sublicensee, to the extent then available to Neurocrine, sufficiently in advance (no fewer than [**] in advance) of the date(s) on which such information is due to the relevant Inbound Licensor under an In-License Agreement to avoid a breach of such In-License Agreement. All payments owed by Voyager under the In-License Agreements, including license fees, royalties and milestones, shall be allocated between the Parties as set forth in Section 5.2.4 and such payment shall be remitted to the applicable Inbound Licensor by Voyager. Notwithstanding anything to the contrary in this Agreement, unless otherwise agreed by the applicable counterparty, the provisions regarding currency conversion, international payments and late payments, and other relevant definitions and provisions, of the relevant In-License Agreements shall apply to calculate the payments due under the relevant In-License Agreements (but not the payments due under this Agreement).

8.7 Accounting; Audit.

8.7.1 Each Party (the "Payor") agrees to keep, and to require its Affiliates and Sublicensees to keep, full, clear and accurate records for a minimum period of [**] after the relevant payment is owed pursuant to this Agreement, setting forth as applicable the sales and other disposition of Products sold or otherwise disposed of, the Development and Commercialization activities with respect to Products, and the Development Costs incurred therewith (including as specified in Section 2.2.2), in sufficient detail to enable royalties and compensation payable to, or the Development Costs payable by, the other Party (the "Payee") hereunder to be determined.

8.7.2 Neurocrine agrees, upon not less than [**] prior written notice, to permit, and to require its Affiliates to permit, such books and records relating to such Products to be examined by an independent accounting firm selected by Voyager and reasonably acceptable to Neurocrine for the purpose of verifying reports provided (or required to be provided) by Neurocrine under this ARTICLE 8 or under the Co-Co Agreement. Voyager agrees, upon not less than [**] prior written notice, to permit, and to require its Affiliates to permit, such books and records relating to Development Costs and other costs under the Co-Co Agreement to be examined by an independent accounting firm selected by Voyager and reasonably acceptable to Neurocrine for the purpose of verifying reports and invoices provided (or required to be provided) by Voyager under Section 2.2.2 or under the Co-Co Agreement. Any such audit shall not be performed more frequently than [**] period, shall not audit any previously audited records, and shall be conducted under appropriate confidentiality provisions, for the sole purpose of verifying the accuracy and completeness of all financial, accounting and numerical information and calculations provided under this Agreement or under the Co-Co Agreement. The independent accounting firm shall only share the results of the audit, not the underlying records, with the auditing party.

8.7.3 Any audit conducted by Voyager is to be made at the expense of Voyager, except if the results of the audit reveal an underpayment of royalties, milestones or other payments to Voyager under this Agreement or under the Co-Co Agreement of [**] percent ([**]%) or more in the audited period, in which case: (a) Neurocrine shall promptly remit to Voyager the amount of such underpayment; and (b) the reasonable fees and expenses for such audit shall be paid by Neurocrine. Any audit conducted by Neurocrine is to be made at the expense of Neurocrine, except if the results of the audit reveal an overpayment of Development Costs or other payments to Voyager under this Agreement or under the Co-Co Agreement of [**] percent ([**]%) or more in the audited period, in which case: (x) Voyager shall promptly remit to Neurocrine the amount of such overpayment; and (y) the reasonable fees and expenses for such audit shall be paid by Voyager. Any audit that reveals an underpayment or overpayment, as the case may be, of less than [**] percent ([**]%) in the audited period, shall be made at the expense of the Party conducting the audit.

8.8 Currency Conversion. When calculating Net Sales, the amount of such sales or costs in foreign currencies shall be converted into Dollars using the standard methodologies employed by Neurocrine generally for consolidation purposes. Neurocrine shall provide reasonable documentation of the calculation and reconciliation of the conversion figures on a Product-by-Product and country-by-country basis as part of its report of Net Sales for the period covered under the report.

8.9 Books and Records. Any books and records to be maintained under this Agreement by a Party or its Affiliates or Sublicensees shall be maintained in accordance with GAAP.

8.10 Methods of Payments. All payments due from one Party to the other Party under this Agreement shall be paid in Dollars by wire transfer to a bank in the United States designated in writing by the Payee.

8.11 Taxes.

8.11.1 Each Party shall be solely responsible for the payment of all taxes imposed on its share of income arising directly or indirectly from the activities of the Parties under this Agreement.

8.11.2 In the event that Neurocrine is required to withhold any tax to be paid to, or held for the benefit of, the tax or revenue authorities in any country in the Territory regarding any payment to Voyager, such amount shall be deducted from the payment to be made by Neurocrine; provided that Neurocrine shall take reasonable and lawful actions to avoid and minimize such withholding and promptly notify Voyager so that Voyager may take lawful actions to eliminate or minimize such withholding. Neurocrine shall promptly furnish Voyager with copies of any tax certificate or other documentation evidencing such withholding, as necessary, to enable Voyager to support a claim, if permissible, for income tax credit or refund in respect of any amount so withheld. Each Party shall cooperate with the other Party in claiming exemptions from such deductions or withholdings under any agreement or treaty in effect from time to time. The Parties shall use commercially reasonable efforts to reduce or eliminate such withholding, including providing any reasonable documentation to reduce or eliminate such withholding.

8.11.3 If a withholding or deduction obligation arises as a result of any action by Neurocrine (including any assignment, sublicense, change of place of incorporation, or failure to comply with applicable Laws or filing or record retention requirements) (a "Withholding Tax Action"), then the sum payable by Neurocrine (in respect of which such deduction or withholding is required to be made) shall be increased to the extent necessary to ensure that Voyager receives a sum equal to the sum which it would have received had no such Withholding Tax Action occurred.

8.11.4 No Partnership. Nothing contained in this Agreement shall be deemed or construed by the Parties, any of their Affiliates or any Third-Party to treat the relationship between the Parties contemplated by this Agreement as a partnership, joint venture or other business entity under Treasury Regulations Section 301.7701-1(a)(2) (or any corresponding provision under state, local or non-U.S. tax law) (an "Entity"). No Party (or successor or assignee) intends, for tax purposes, on reporting the relationships established by this Agreement as an Entity, including either: (a) making any disclosure that the relationships established by this Agreement may give rise to an Entity (whether on a U.S. Internal Revenue Service Form 8275 or otherwise); or (b) withholding any amounts from payments made to the other Party pursuant to Section 1446 of the Code (or any corresponding provision under state, local or non-U.S. tax law), unless required by a Governmental Authority on audit or other examination. Notwithstanding the foregoing, if the arrangement between the Parties as contemplated by this Agreement is determined to constitute an Entity under Applicable Law (as determined based on the opinion (on a "should" basis) of a nationally recognized law or accounting firm) or by a Governmental Authority on audit or other examination, the Party that is aware of such determination shall provide notice to the other Party regarding such treatment and the Parties will reasonably cooperate with one another to satisfy any tax filing or reporting obligation arising as a result of such determination, including by providing any information, forms or other certifications necessary to satisfy such obligations.

8.11.5 Other Taxes and Cooperation.

(a) To the extent applicable in respect of Other Taxes, all payments or amounts due under this Agreement, whether monetary or non-monetary, are exclusive of Other Taxes. In accordance with Section 8.11.5(b) below, the applicable Party responsible for Other Taxes will timely pay any such Other Taxes that are properly chargeable with respect to the transactions governed by this Agreement. Upon request by the receiving Party, the supplying Party will provide an invoice (or equivalent document) to support the charge for Other Taxes due with respect to the supply.

(b) Voyager, on the one hand, and Neurocrine, on the other hand, shall each pay all Other Taxes and fees (including any penalties and interest) incurred in connection with this Agreement and for which such Party is responsible under applicable Law. The Parties will provide all information that the other reasonably requests in respect of its payment of Other Taxes to assist each other in recovering such Other Taxes, as applicable.

(c) To the extent any supply of goods or services under this Agreement will be taxed in accordance with prevailing legislation in respect of Other Taxes, the Parties will reasonably cooperate to enable the use of any exemptions, suspensions or other reliefs to the extent reasonably practicable.

8.12 Late Payments. Any undisputed amount owed by Neurocrine to Voyager under this Agreement that is not paid on or before the date such payment is due shall bear simple interest at a rate per annum equal to the lesser of: (a) the greater of (i) the prime or equivalent rate per annum quoted by *The Wall Street Journal* on the first Business Day after such payment is due, plus [**], or (ii) [**] percent ([**]%) per month; or (b) the highest rate permitted by applicable Law, calculated on the number of days such payments are paid after such payments are due.

ARTICLE 9
EXCLUSIVITY

9.1 Exclusivity.

9.1.1 Voyager. During the Term of this Agreement, neither Voyager nor any of its Affiliates shall, except as otherwise permitted in this ARTICLE 9, either alone or with or for any Third-Party, Develop, Manufacture or Commercialize any Competitive Product or grant any Affiliate or Third-Party a license or sublicense to enable any Third-Party to do so.

9.1.2 Neurocrine. During the Term of this Agreement, neither Neurocrine nor any of its Affiliates shall, except as otherwise permitted in this ARTICLE 9, either alone or with or for any Third-Party, Develop, Manufacture or Commercialize any Competitive Product for which the viral vector is AAV or grant any Affiliate or Third-Party a license or sublicense to do so.

9.2 Exception for Basic Research. Notwithstanding Section 9.1, Neurocrine and Voyager shall be free during the Term, either alone or with or for an Affiliate or a Third-Party, to conduct basic scientific, non-clinical and pre-clinical Development with respect to the biological mechanism of action, pharmacology, structure-activity relationship (SAR) or the like for any Gene Therapy Product; provided, however, that Voyager shall not conduct any basic scientific, non-clinical and pre-clinical Development with respect to any Gene Therapy Product directed to a

Program Target, including a Collaboration Candidate or Product, other than under a Development Plan or Co-Co Agreement, without the prior written approval of the JSC, and the conduct of such non-clinical and pre-clinical Development shall be subject to the supervision and oversight of the JSC.

9.3 Acquisitions.

9.3.1 If (x) during the term of the exclusivity covenant in Section 9.1, a Party or any of its Affiliates (such Party, the "Acquisition Party") acquires or is acquired by a Third-Party (an "Acquired Affiliate") (whether such acquisition occurs by way of a purchase of assets, merger, consolidation, change of control or otherwise) that is, at the time of such acquisition, engaging in any activities that would violate Section 9.1.1or 9.1.2, as applicable, if conducted by such Acquisition Party (such activities, an "Acquired Competing Program" and any product Developed, Commercialized or otherwise Exploited thereunder, an "Acquired Competing Product"), then (y) the Acquisition Party or its Acquired Affiliate shall, no later than [**] following the date of consummation of the relevant acquisition, notify the other Party in writing that the Acquisition Party or such Acquired Affiliate has elected one of the following:

(a) to divest, whether by license or otherwise, its interest in the Acquired Competing Program to a Third-Party, to the extent necessary to be in compliance with Section 9.1, with no rights in such Acquired Competing Program retained by the Acquisition Party or any of its Affiliates;

(b) to terminate Development, Manufacture and Commercialization under the Acquired Competing Program, to the extent necessary to be in compliance with Section 9.1;

(c) if the Acquisition Party is Voyager and Voyager is acquired by a Third Party prior to the end of the Discovery Period, to permit Neurocrine to elect (in Neurocrine's sole discretion) to terminate Voyager's Discovery Activities under any then current Programs; or

(d) if the Acquisition Party is Voyager and Voyager is acquired by a Third Party after the end of the Discovery Period, to permit Neurocrine to elect (in Neurocrine's sole discretion) that any or all of the consequences under Section 15.5.2 apply.

9.3.2 If the Acquisition Party or its Acquired Affiliate notifies the other Party in writing that it intends to divest such Acquired Competing Program or terminate Development, Manufacture and Commercialization under the Acquired Competing Program as provided in Section 9.3.1(a) or 9.3.1(b), then the Acquisition Party or its Acquired Affiliate, as applicable, shall effect the consummation of such divestiture within [**] or effect such termination within [**] after the consummation of the relevant acquisition, subject to compliance with applicable Law, and shall confirm to the other Party in writing when such divestiture or termination has been completed. The Acquisition Party shall keep the other Party reasonably informed of its and its Affiliates' efforts and progress in effecting such divestiture or termination until it is completed. Until such divestiture or termination occurs, the Acquisition Party shall keep its and its Affiliates' activities with respect to such Acquired Competing Program separate from their activities under this Agreement or any Co-Co Agreement.

9.3.3 If the Acquisition Party is Voyager and Voyager notifies Neurocrine in writing that it elects for Neurocrine to have the right to terminate Voyager's Discovery Activities under any then current Programs under Section 9.3.1(c), and if Neurocrine elects such termination, then Voyager shall provide (at Voyager's expense) technology transfer and licenses necessary for Neurocrine to complete any currently contemplated Voyager Discovery Activities through the end of the Discovery Period.

9.3.4 If the Acquisition Party is Voyager and Voyager notifies Neurocrine in writing that it elects either Section 9.3.1(c) or Section 9.3.1(d), then Voyager and the Acquired Affiliate shall keep all activities with respect to such Acquired Competing Program separate from activities under this Agreement or any Co-Co Agreement, including by ensuring that (a) no personnel involved in any Exploitation of the Acquired Competing Product have access to non-public plans or non-public information relating to the Exploitation of any Collaboration Candidate or Product or any Confidential Information of Neurocrine; and (b) no Voyager IP or Neurocrine IP is used in connection with the Exploitation of the Acquired Competing Product. For clarity, Section 15.5 will continue to apply upon any Change of Control of Voyager, to the extent applicable. If the Acquisition Party is Voyager and Voyager notifies Neurocrine in writing that it elects either Section 9.3.1(c) or Section 9.3.1(d), and Neurocrine fails to elect to terminate Voyager's Discovery Activities under any then current Programs or for the consequences of Section 15.5.2 to apply, as applicable, then the continuation of the Acquired Competing Program shall not be deemed a breach of Section 9.1.1.

9.3.5 Subject to the Acquisition Party's compliance with this Section 9.3, the activities of the Acquisition Party or its Acquired Affiliate with respect to any Acquired Competing Program shall not be a breach of this Agreement.

ARTICLE 10
INTELLECTUAL PROPERTY RIGHTS

10.1 Ownership; Disclosure.

10.1.1 Neurocrine Background IP. As between the Parties, Neurocrine will own and control all right, title and interest in and to all Patent Rights or Know-How: (a) Controlled by Neurocrine and existing as of or before the Effective Date; or (b) Created or acquired solely by or on behalf of Neurocrine (including through its Representatives) after the Effective Date outside of its activities under this Agreement ((a) and (b), collectively, "Neurocrine Background IP").

10.1.2 Voyager Background IP. As between the Parties, Voyager will own and control all right, title and interest in and to all Patent Rights or Know-How: (a) Controlled by Voyager and existing as of or before the Effective Date; or (b) Created or acquired solely by or on behalf of Voyager (including through its Representatives) after the Effective Date outside of its activities under this Agreement ((a) and (b), collectively, "Voyager Background IP").

10.1.3 Arising IP.

(a) Arising IP will be owned as follows: (i) Voyager will solely own all Arising Capsid IP; and (ii) with respect to all Arising IP other than Arising Capsid IP: (A) Voyager

will solely own all such Arising IP Created solely by Representatives of Voyager; (B) Neurocrine will solely own all such Arising IP Created solely by Representatives of Neurocrine; and (C) the Parties will jointly own all such Arising IP Created jointly by Representatives of Neurocrine and Representatives of Voyager ("Joint Arising IP").

(b) Except as expressly provided in this Agreement, each Party may (subject to the licenses and exclusivity provisions of this Agreement) practice the Joint Arising IP, but neither Party may grant licenses or otherwise encumber its ownership interest in any Joint Arising IP without the prior written consent of the other Party.

(c) Neurocrine, on behalf of itself and its Affiliates, hereby assigns, and to the extent such present assignment is not possible, agrees to assign, to Voyager all of Neurocrine's right, title and interest in and to all Arising Capsid IP. Neurocrine will, at its sole cost and expense, provide Voyager all reasonable assistance and cooperation in connection with effecting with the foregoing ownership allocation, including providing any necessary powers of attorney and executing any other required documents or instruments as requested by Voyager.

10.1.4 Disclosure.

(a) During the Term, the Parties shall promptly disclose to each other any Arising IP that Covers or is otherwise necessary for the Development, Manufacture and Commercialization of any Collaboration Candidate or Product.

(b) During the Term, Neurocrine shall promptly disclose to Voyager any Arising Capsid IP made solely by Neurocrine or jointly by the Parties.

(c) During the Term, each Party shall promptly disclose to the other Party any Joint Arising IP of which it becomes aware that is not otherwise captured by Section 10.1.4(a) or 10.1.4(b) above.

10.2 Patent Prosecution and Maintenance.

10.2.1 Voyager Patent Rights. Subject to the terms of any applicable In-License Agreement and Co-Co Agreement, and except as set forth in Section 10.2.2 and 10.2.3 below, Voyager shall have the sole right, at its sole cost and cost and expense, for Prosecuting and Maintaining the Voyager Patent Rights and for conducting and defending any Defense Proceeding relating thereto. Notwithstanding anything herein to the contrary, Voyager shall not include any data or information related to any Collaboration Candidate (other than related solely to the Voyager Capsid therein) or Program Target (or any non-human homolog thereof) in any Voyager Patent Rights (or disclose any such data or information in connection with the Prosecution and Maintenance thereof) without Neurocrine's prior written consent, which Neurocrine may grant or withhold in its sole discretion.

10.2.2 Program Capsid Patent Rights.

(a) Subject to the terms of any applicable In-License Agreement and Co-Co Agreement:

(i) Subject to Section 10.2.3, Voyager shall have the first right, at its sole cost and expense, for Prosecuting and Maintaining the Program Capsid Patent Rights and for conducting and defending any opposition, reexamination request, nullity action, interference, or other post-grant proceeding involving an attack upon the validity, title or enforceability thereof relating thereto, and for initiating any interference, including in each case any appeals therefrom (each, a "Defense Proceeding") (except that in connection with any actions subject to Section 10.3, the Party with responsibility for such action pursuant to Section 10.3 shall have responsibility for any related Defense Proceedings). Upon request by Neurocrine, the Parties shall coordinate and use reasonable efforts, in connection with Voyager's Prosecution and Maintenance of the Program Capsid Patent Rights, to enable Neurocrine to file patent applications, including divisionals, continuations or other patent applications for Voyager Product-Specific Patent Rights in accordance with Section 10.2.3.

(ii) Voyager shall keep Neurocrine fully informed with respect to: (A) the issuance of a Program Capsid Patent Right being Prosecuted and Maintained by Voyager pursuant to this Section 10.2.2; and (B) the abandonment of any Program Capsid Patent Right.

(iii) Without limiting the foregoing, Voyager shall: (A) provide Neurocrine with copies of the text of the applications for any Program Capsid Patent Right as soon as practicable but at least [**] before filing, except for urgent filings, in which case Voyager shall provide copies as soon as practicable before, simultaneously with or immediately after filing; (B) provide Neurocrine with a copy of each submission made to and material or substantive document received from a patent authority, court or other tribunal regarding any Program Capsid Patent Right reasonably promptly after making such filing or receiving such document, including a copy of each application as filed together with notice of its filing date and application number; (C) keep Neurocrine advised of the status of all substantive communications, actual and prospective filings or submissions regarding any Program Capsid Patent Right, and give Neurocrine copies of any such communications, filings and submissions proposed to be sent to any patent authority or judicial body; (D) consider in good faith and reasonably incorporate Neurocrine's comments on such communications, filings and submissions for any Program Capsid Patent Right unless incorporating such comments would reasonably be expected to have a material adverse effect on the scope of any Program Capsid Patent Right that Covers products being developed or commercialized by Voyager that are not Collaboration Candidates; and (E) file Program Capsid Patent Rights in particular countries in which Neurocrine desires Voyager to file a particular Program Capsid Patent Right, provided, however, that Neurocrine shall reimburse Voyager for all expenses incurred in Prosecuting and Maintaining Program Capsid Patent Rights in countries requested by Neurocrine in which a company similarly situated to Voyager may not file patent applications in accordance with commercially reasonable business practices. Neurocrine's rights pursuant to this Section 10.2.2(a)(iii) shall terminate with respect to Program Capsid Patent Rights that are relevant to one Program only at such time as such Program is terminated pursuant to the terms of this Agreement.

10.2.3 Voyager Product-Specific Patent Rights.

(a) Subject to the terms of any applicable In-License Agreement and Co-Co Agreement:

(i) Neurocrine shall have the first right, at its sole cost and expense, for Prosecuting and Maintaining the Voyager Product-Specific Patent Rights and for conducting any Defense Proceeding relating thereto (except that in connection with any actions subject to Section 10.3, the Party with responsibility for such action pursuant to Section 10.3 shall have responsibility for any related Defense Proceedings).

(ii) Neurocrine shall keep Voyager fully informed with respect to: (A) the issuance of a Voyager Product-Specific Patent Right being Prosecuted and Maintained by Neurocrine pursuant to this Section 10.2.3(a); and (B) the abandonment of any Voyager Product-Specific Patent Right Prosecuted and Maintained by Neurocrine pursuant to this Section 10.2.3(a).

(iii) Without limiting the foregoing, Neurocrine shall: (A) provide Voyager with copies of the text of the applications for any Voyager Product-Specific Patent Right it Prosecutes or Maintains as soon as practicable but at least [**] before filing, except for urgent filings, in which case Neurocrine shall provide copies as soon as practicable before, simultaneously with or immediately after filing; (B) provide Voyager with a copy of each submission made to and material or substantive document received from a patent authority, court or other tribunal regarding any Voyager Product-Specific Patent Right reasonably promptly after making such filing or receiving such document, including a copy of each application as filed together with notice of its filing date and application number; (C) keep Voyager advised of the status of all substantive communications, actual and prospective filings or submissions regarding any Voyager Product-Specific Patent Right, and give Voyager copies of any such communications, filings and submissions proposed to be sent to any patent authority or judicial body; and (D) consider in good faith Voyager's comments on such communications, filings and submissions for any such Voyager Product-Specific Patent Right and shall reasonably incorporate such comments unless their incorporation would reasonably be expected to have a material adverse effect on the scope of any Voyager Product-Specific Patent Right.

(iv) Notwithstanding Section 10.2.3(a)(i) Neurocrine shall not file any Voyager Product-Specific Patent Right or any other Patent Right disclosing a Voyager Capsid prior to the first publication of any Capsid Patent Right that first discloses the sequence for the corresponding Voyager Capsid that is the subject of the corresponding Collaboration Candidate or Product, without first receiving Voyager's written approval, not to be unreasonably withheld, conditioned or delayed, to make such filing. In addition to other provisions that the Parties may agree are appropriate to implement, in the event that: (A) an application for Patent Rights disclosing a Voyager Capsid whose sequence has not been publicly disclosed and that is not owned by Voyager as a Voyager Product-Specific Patent Right is filed after Voyager's approval in accordance this Section 10.2.3(b); or (B) any other Patent Rights (e.g., Joint Patent Rights) filed by Neurocrine creates an obviousness-type double patenting (OTDP) rejection or challenge against a Capsid Patent Right and that requires filing of a terminal disclaimer to obviate such rejection or challenge (and cannot otherwise be overcome by other approaches as agreed to by the Parties), Neurocrine will assign its right, title, and interest in such Patent Rights to Voyager in the United States only, subject to Neurocrine receiving the exclusive license set forth in Section 3.1.2(b); provided that Neurocrine will retain the sole right, at its sole cost and expense: (x) to Prosecute

and Maintain the Patent Rights in all countries; and (y) for enforcing or defending all such assigned Voyager Patent Rights.

(b) Neurocrine shall notify Voyager as to any decision to abandon, to cease Prosecution and Maintenance of, or not to continue to pay the expenses of Prosecution and Maintenance of, any Voyager Product-Specific Patent Right in any country in which it was filed. Neurocrine will provide such notices at least [**] prior to any filing or payment due date, or any other due date that requires action, in connection with such Voyager Product-Specific Patent Right. Notwithstanding the foregoing, if Neurocrine has provided notice of termination under Section 14.2, Neurocrine will not discontinue the Prosecution and Maintenance of any Voyager Product-Specific Patent Right during the [**] notice period until such Prosecution and Maintenance is assumed by Voyager pursuant to this Section 10.2.3(b); provided that Voyager will be responsible for all Out-of-Pocket Costs incurred by Neurocrine to conduct any Prosecution and Maintenance activities during such notice period that are requested by Voyager. The Parties agree that, promptly after notice of termination is provided under Section 14.2, the Collaboration IP Working Group will meet and determine a plan for the orderly transition of such Prosecution and Maintenance to Voyager, with the good faith objective of transitioning Prosecution and Maintenance to Voyager within [**].

10.2.4 Neurocrine Patent Rights. Neurocrine shall be responsible, at its sole cost and expense, and shall have the exclusive right, but not the obligation, for Prosecuting and Maintaining the Neurocrine Patent Rights and for conducting Defense Proceedings relating thereto.

10.2.5 Joint Patent Rights.

(a) Subject to the terms of the Co-Co Agreement, if applicable:

(i) Subject to Section 10.2.5(b), Neurocrine shall have the first right, at its sole cost and expense, for Prosecuting and Maintaining in both Parties' names the Joint Patent Rights and for conducting any Defense Proceeding relating thereto. Voyager shall execute any powers of attorney necessary for Neurocrine's counsel to conduct such activities.

(ii) Neurocrine shall keep Voyager fully informed with respect to (A) the issuance of any Joint Patent Right being Prosecuted and Maintained by Neurocrine pursuant to this Section 10.2.5(a), and (B) the abandonment of any Joint Patent Right being Prosecuted and Maintained by Neurocrine pursuant to this Section 10.2.5(a).

(iii) Without limiting the foregoing, Neurocrine shall: (A) provide Voyager with copies of the text of the applications for any such Joint Patent Right as soon as practicable but at least [**] before filing, except for urgent filings, in which case Neurocrine shall provide copies as soon as practicable before, simultaneously with or immediately after filing; (B) provide Voyager with a copy of each submission made to and material or substantive document received from a patent authority, court or other tribunal regarding any such Joint Patent Right reasonably promptly after making such filing or receiving such material document, including a copy of each application as filed together with notice of its filing date and application number; (C) keep Voyager advised of the status of all substantive communications, actual and prospective

filings or submissions regarding any such Joint Patent Right, and shall give Voyager copies of any such communications, filings and submissions proposed to be sent to any patent authority or judicial body; and (D) consider in good faith Voyager's comments on such communications, filings and submissions for any such Joint Patent Right.

(b) Neurocrine shall notify Voyager as to any decision to abandon, to cease Prosecution and Maintenance of, or not to continue to pay the expenses of Prosecution and Maintenance of, any Joint Patent Right in any country in which it was filed. Neurocrine shall provide such notices at least [**] prior to any filing or payment due date, or any other due date that requires action, in connection with such Joint Patent Right. Thereafter, Voyager may, upon written notice to Neurocrine, in both Parties' names and at Voyager's sole cost and expense, control the Prosecution and Maintenance of such Joint Patent Right, and Voyager shall keep Neurocrine reasonably informed of the status of such Joint Patent Right in accordance with Sections 10.2.5(a)(ii) and (iii), *mutatis mutandis*.

(c) The Parties shall undertake reasonable efforts and cooperate to ensure to the fullest extent practicable and not prejudicial that Joint Patent Rights are Prosecuted and Maintained in a manner that separates the claims pertaining to one Program and the Collaboration Candidates and Products arising therefrom, on the one hand, from other Programs and the Collaboration Candidates and Products arising therefrom, on the other hand, into distinct patent applications and ultimately separate issued patents.

10.2.6 Cooperation. Each Party shall reasonably cooperate with and assist the other Party in connection with the activities of such Party under Section 10.2 upon the reasonable request of the other Party, including by making scientists and scientific records reasonably available and the execution of all such documents and instruments and the performance of such acts as may be reasonably necessary in order to permit the other Party to continue any Prosecution or Maintenance of the applicable Patent Rights.

10.2.7 Patent Term Extension. Notwithstanding anything to the contrary in Section 10.2.1, 10.2.3 or 10.2.5, the Collaboration IP Working Group shall discuss all decisions regarding patent term extensions in the Territory, including in the United States with respect to extensions pursuant to 35 U.S.C. § 156 *et. seq.* and in other jurisdictions pursuant to supplementary protection certificates, and in all jurisdictions with respect to any other extensions that are now or become available in the future, wherever applicable, for Voyager Product-Specific Patent Rights and the Joint Patent Rights, in each case including whether or not to so apply and which Party shall so apply; provided that Neurocrine shall have the right to make all decisions with respect to any such extension of a Voyager Product-Specific Patent Right or Joint Patent Right Covering any Product; provided that Neurocrine shall not have the right to extend any Voyager Product-Specific Patent Right that Voyager intends to extend with respect to a different product for which there is no other Patent Right reasonably available to extend. Each Party shall provide prompt and reasonable assistance, as requested by the other Party, including by taking such action as is required under any applicable Law to obtain such extension or supplementary protection certificate.

10.3 Enforcement and Defense. Subject to the terms of any applicable In-License Agreement and any applicable Co-Co Agreement:

10.3.1 Notice. Each Party shall promptly notify the other of any knowledge it acquires of any: (a) actual or potential infringement by a Third-Party of any Voyager Patent Right, Neurocrine Patent Right or Joint Patent Right that is or would be competitive with a Collaboration Candidate or Product; or (b) submission to a Party or a Regulatory Authority of an application for a product (including an application under Section 351(k) of the PHSA) that references a Product (each of (a) and (b), a "Competitive Infringement"); or (c) actual or potential infringement, other than a Competitive Infringement, by a Third-Party of any Voyager Patent Right, Neurocrine Patent Right or Joint Patent Right by the manufacture, use or sale of a product that includes a Program Capsid; or (d) submission to a Party or a Regulatory Authority of an application for a product (including an application under Section 351(k) of the PHSA) that references a product (other than a Product) that includes a Program Capsid.

10.3.2 Actions.

(a) If any Neurocrine Patent Right is infringed by a Third-Party in any country in the Territory, then Neurocrine shall have the sole right, but not the obligation, to institute and control any action or proceeding with respect to such infringement of such Patent Right, by counsel of its own choice.

(b) If any Capsid Patent Right that is not a Voyager Patent Right is infringed by a Third-Party in any country in the Territory, then Voyager shall have the sole right, but not the obligation, to institute and control any action or proceeding with respect to such infringement of such Patent Right, by counsel of its own choice. If, in any such proceeding brought by Voyager, Neurocrine is required to join for standing purposes or in order for Voyager to commence or continue such proceeding, then Neurocrine shall join such proceeding, at Voyager's expense, and shall be represented in such proceeding by counsel of Voyager's choice at Voyager's expense, unless Neurocrine elects to be represented by counsel of its own choice at Neurocrine's expense.

(c) Voyager shall have the primary right, but not the obligation, to institute, prosecute, and control any action or proceeding with respect to Competitive Infringement of any Voyager Patent Right that is not a Voyager Product-Specific Patent Right, by counsel of its own choice, provided that Voyager shall not unreasonably refuse to accept input from Neurocrine with respect to such proceeding, and Neurocrine shall have the right to be represented in such proceeding by counsel of Neurocrine's choice at Neurocrine's expense. If in any such proceeding brought by Voyager, Neurocrine is required to join for standing purposes or in order for Voyager to commence or continue any such proceeding, then Neurocrine shall join such proceeding, at Voyager's expense, and shall be represented in such proceeding by counsel of Neurocrine's choice at Neurocrine's expense. If Voyager does not bring an infringement action pursuant to this Section 10.3.2(c) within [**] after receipt of notice of the existence of an infringement (or in cases where there is a relevant statutory period during which an infringement action must be commenced or in which any material rights may be lost that would expire prior to the expiration of such [**] period and of which Neurocrine has notified Voyager promptly after it becomes aware, [**] prior to the expiration of such relevant statutory period), Voyager and Neurocrine shall meet and discuss Voyager's reasons for not initiating a lawsuit or otherwise making or prosecuting a claim. If following such discussions Neurocrine desires to initiate a lawsuit or otherwise make or prosecute a claim with respect to the Competitive Infringement and so notifies Voyager in writing, then,

upon receiving Voyager's prior written consent (unless Voyager does not have such consent right as provided in the following sentence), which shall not be unreasonably withheld, conditioned or delayed, Neurocrine may institute, prosecute, and control such action; provided that, if, under the terms of an applicable In-License Agreement, Voyager has an applicable enforcement right that it cannot delegate to Neurocrine then, at Neurocrine's request and expense, Voyager shall exercise such rights in such infringement action as directed by Neurocrine. Notwithstanding the foregoing, Voyager shall not have any right of consent pursuant to the immediately preceding sentence if (i) there is no product Covered by the applicable Voyager Patent Rights then being Commercialized by Voyager or its Affiliate or Third-Party licensee, or (ii) the applicable Product no longer has Regulatory Exclusivity and Neurocrine notifies Voyager in writing that Neurocrine has determined in good faith that an action or proceeding with respect to such Voyager Patent Rights should be instituted to enable continued market exclusivity for such Product. If Voyager has a right of consent and does not consent to Neurocrine's instituting such action, then the applicable Voyager Patent Right will be considered not to exist for purposes of any Royalty Term or royalty adjustments in Section 8.5, and no further Milestone Payments shall be payable or paid with respect to the applicable Product Covered by such Voyager Patent Right unless such Product is also Covered by any Voyager Patent Rights other than the Voyager Patent Rights for which Voyager does not provide such consent. If in any such proceeding Voyager is required to join for standing purposes or in order for Neurocrine (or an Inbound Licensor) to commence or continue any such proceeding, then Voyager shall join such proceeding, at Neurocrine's expense, and shall be represented in such proceeding by counsel of Voyager's choice at Voyager's expense.

(d) Neurocrine shall have the primary right, but not the obligation, to institute, prosecute, and control any action or proceeding with respect to Competitive Infringement of any Voyager Product-Specific Patent Right or Joint Patent Right, by counsel of its own choice, provided that Neurocrine shall not unreasonably refuse to accept input from Voyager with respect to such proceeding. If in any such proceeding brought by Neurocrine, Voyager is required to join for standing purposes or in order for Neurocrine to commence or continue any such proceeding, then Voyager shall join such proceeding, at Neurocrine's expense, and shall be represented in such proceeding by counsel of Voyager's choice at Voyager's expense. The exercise by Neurocrine of the right to bring an infringement action shall be subject to and consistent with the terms of all applicable In-License Agreements; provided that, if, under the terms of an applicable In-License Agreement, Voyager has an applicable enforcement right that it cannot delegate to Neurocrine then, at Neurocrine's request and expense, Voyager shall exercise such rights in such infringement action as directed by Neurocrine. If Neurocrine does not bring an infringement action pursuant to this Section 10.3.2(d) within [**] after receipt of notice of the existence of an infringement (or in cases where there is a relevant statutory period during which an infringement action must be commenced or in which any material rights may be lost that would expire prior to the expiration of such [**] period and of which Voyager has notified Neurocrine promptly after it becomes aware, [**] prior to the expiration of such relevant statutory period), Voyager and Neurocrine shall meet and discuss Neurocrine's reasons for not initiating a lawsuit or otherwise making or prosecuting a claim. If following such discussions Voyager desires to initiate a lawsuit or otherwise make or prosecute a claim with respect to the Competitive Infringement and so notifies Neurocrine in writing, then upon receiving Neurocrine's prior written consent, which consent shall not be unreasonably withheld, Voyager may institute, prosecute, and control such action. If in any such proceeding Neurocrine is required to join for standing purposes or in order for Voyager (or an

Inbound Licensor) to commence or continue any such proceeding, then Neurocrine shall join such proceeding, at Voyager's expense, and shall be represented in such proceeding by counsel of Neurocrine's choice at Neurocrine's expense.

(e) The Party initiating the suit shall have the sole and exclusive right to elect counsel for any suit initiated by it pursuant to Section 10.3.2(a), (c) or (d); provided that, with respect to a Voyager Product-Specific Patent Right or Joint Patent Right, such counsel is reasonably acceptable to the other Party.

(f) Each Party agrees to cooperate fully in any action under this Section 10.3.2 that is controlled by the other Party, including executing legal papers and cooperating in the prosecution as may be reasonably requested by the controlling Party, all at the controlling Party's expense. Neither Party will separately or sequentially enforce, and each Party will ensure that its Affiliate and licensee does not separately or sequentially enforce, any Patent Rights under this Section 10.3.2 that are subject to a terminal disclaimer.

(g) Unless otherwise agreed by the Parties in writing, and subject to the terms of the Co-Co Agreement, the amount of any recovery from a proceeding brought under this Section 10.3.2 shall first be applied to the Out-of-Pocket Cost of such action incurred by the Party prosecuting the applicable action, and any remaining recovery amount shall be applied to the Out-of-Pocket Cost of such action incurred by the other Party (if any), and then, of the remaining amount, (i) any recovery for a proceeding brought by Neurocrine with respect to a Voyager Patent Right or Joint Patent Right or by Voyager with respect to a Voyager Patent Right (other than a Voyager Product-Specific Patent Right) shall be retained by Neurocrine, but shall be deemed Net Sales of the applicable Product in the applicable country and subject to royalty payments under Section 8.3 or, with respect to Co-Co Products, shared equally between the Parties, (ii) any recovery for a proceeding brought by Voyager with respect to a Voyager Product-Specific Patent Right or Joint Patent Right shall be allocated [**] percent ([**]%) to Voyager and [**] percent ([**]%) to Neurocrine and (iii) any recovery for a proceeding brought with respect to a Neurocrine Patent Right shall be retained by Neurocrine. If, in connection with a proceeding brought under this Section 10.3.2 with respect to a Voyager Product-Specific Patent Right, an Inbound Licensor is entitled to a portion of any recovery that is greater than the portion of the recovery payable, after costs, to Voyager, the Parties will meet and agree in good faith on an alternative sharing of such recovery to that set forth in the immediately preceding sentence that takes into account the amounts payable to the applicable Inbound Licensor and results in an equitable allocation of the remaining amounts to Neurocrine and Voyager after payment of such amounts to the applicable Inbound Licensor.

10.3.3 Defense. With respect to any defense or declaratory judgment actions relating to, or other attack upon, validity or enforceability of a Voyager Patent Right, Neurocrine Patent Right or Joint Patent Right that is not a Defense Proceeding, the Party with responsibility for the Prosecution and Maintenance of such Patent Right shall have the first right, but not the obligation, to assume the defense thereof at its sole cost and expense, except that if such action is in connection with a Competitive Infringement, Section 10.3.2 will apply to such action (as if it were enforcement against a Competitive Infringement).

10.4 Infringement Claimed by Third Parties.

10.4.1 If a Third-Party commences, or threatens to commence, any proceeding against a Party alleging infringement of such Third-Party's intellectual property by the Exploitation by a Party, its Affiliates, subcontractors or Sublicensees of any Collaboration Candidate or Product, the Party against whom such proceeding is threatened or commenced shall give prompt notice to the other Party.

10.4.2 Unless the Party against whom such proceeding is filed seeks indemnification for a claim covered pursuant to ARTICLE 13, such Party shall, as between the Parties, have the sole right to control the defense and settlement of any such proceeding under Section 10.4.1 at its own cost.

10.5 Marking. Neurocrine and its Affiliates and Sublicensees shall mark each Product in such a manner to conform with the patent laws and practice of any country in which such Product is Manufactured or sold or to which such Product is shipped to ensure maximum enforceability of Patent Rights in such country.

10.6 Trademarks. Except for Products arising from the GBA1 Program if Voyager exercises its Co-Co Option for such Program, Neurocrine shall have the right to brand Products in the Territory using Neurocrine-related trademarks and any other trademarks and trade names it determines appropriate, which may vary by country or within a country ("Neurocrine Product Marks"). Neurocrine shall own all rights in the Neurocrine Product Marks and, as between the Parties, shall have the sole right to register, maintain, enforce and defend the Neurocrine Product Marks, at its sole expense, provided that Neurocrine will provide Voyager appropriate licenses to the Neurocrine Product Marks under any applicable Co-Co Agreement to undertake activities assigned to Voyager thereunder so requiring such licenses. If Voyager exercises its Co-Co Option for the GBA1 Program, branding of Co-Co Products arising from the GBA1 Program shall be governed by the applicable provisions of the applicable Co-Co Agreement and subject to final review and approval of the JSC.

ARTICLE 11
CONFIDENTIALITY

11.1 Confidentiality; Exceptions. Except to the extent expressly authorized by this Agreement, or as otherwise agreed in writing, the Parties agree that the receiving Party (the "Receiving Party"): (a) shall keep confidential and shall not publish or otherwise disclose; and (b) shall not use for any purpose other than as provided for in this Agreement (which purpose includes exercising its rights and performing its obligations under this Agreement); in each case ((a) and (b)) any Know-How or other confidential and proprietary information and materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise) that is disclosed to it by the other Party (the "Disclosing Party"), including trade secrets, Know-How, Inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the Disclosing Party's past, present or future marketing, financial, or Exploitation activities of any product or potential product or useful technology of the Disclosing Party or the pricing thereof (collectively, "Confidential Information" of the Disclosing Party), except that "Confidential Information" shall exclude information to the extent that it can be established by the Receiving Party that such information:

11.1.1 was in the lawful knowledge and possession of the Receiving Party without restriction on use or disclosure prior to the time it was first disclosed to the Receiving Party by the Disclosing Party, or was otherwise developed independently by the Receiving Party without reference to any of the Disclosing Party's Confidential Information, as evidenced by written records kept in the ordinary course of business, or other documentary proof of actual use by the Receiving Party;

11.1.2 was generally available to the public or otherwise part of the public domain at the time of its first disclosure to the Receiving Party by the Disclosing Party;

11.1.3 became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party by the Disclosing Party and other than through any act or omission of the Receiving Party in breach of this Agreement or the Existing Confidentiality Agreement; or

11.1.4 was lawfully disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third-Party who had no obligation to the Disclosing Party not to disclose such information to others.

Any information disclosed by a Party to the other Party prior to the Execution Date (a) pursuant to the Confidential Disclosure Agreement between Voyager and Neurocrine dated [**] (as amended from time to time, the "Existing Confidentiality Agreement") or (b) solely to the extent relevant to a Program hereunder, pursuant to that certain Collaboration and License Agreement, dated January 28, 2019, by and between Voyager and Neurocrine, as amended from time to time (the "2019 CLA"), in each case ((a) and (b)) that was considered Confidential Information (as defined in the applicable agreement) of a Party shall be Confidential Information of such Party hereunder, subject to the provisions of Sections 11.1.1, 11.1.2, 11.1.3 and 11.1.4. Notwithstanding anything to the contrary, any Capsid Know-How in the Arising Capsid IP that relates to a Voyager Capsid (except to the extent such Capsid Know-How relates to (x) any component of a Collaboration Candidate other than the Voyager Capsid therein; (y) any Program Target or Program Payload; or (z) any method of Manufacture or use of a Collaboration Candidate (and not only the Voyager Capsid therein) or Program Payload) shall be considered the Confidential Information of Voyager, with Voyager considered the Disclosing Party and Neurocrine considered the Receiving Party.

11.2 Authorized Disclosure. Except as expressly provided otherwise in this Agreement, a Receiving Party may disclose Confidential Information of the Disclosing Party as follows: (a) to the extent required to those of its Representatives who reasonably need to know such Confidential Information in order to advise or assist the Receiving Party in connection with the performance of its obligations or exercise of its rights granted or reserved in this Agreement and under appropriate written (or legal or ethical such as in the case of attorneys) confidentiality and non-use obligations no less protective of the Disclosing Party than those set forth in this Agreement; (b) as required by applicable Law; provided, however, that if a Receiving Party is required by Law to make any such disclosure of a Disclosing Party's Confidential Information it will, except where impracticable, give reasonable advance notice to the Disclosing Party of such disclosure requirement, limit disclosure to only the Confidential Information requested to be disclosed and, if requested by the Disclosing Party, cooperate with the Disclosing Party to secure confidential treatment of such Confidential Information required to be disclosed; (c) in communication with existing or bona fide

prospective investors, lenders, professional advisors, acquirers, merger partners, subcontractors, licensees, collaborators or Inbound Licensors on a need to know basis, in each case under appropriate written (or legal or ethical such as in the case of attorneys) confidentiality and non-use obligations substantially equivalent to those of this Agreement, except that the term of such obligations may be shorter, and with respect to any disclosure to an Inbound Licensor under an Existing In-License Agreement, Neurocrine acknowledges that the relevant Inbound Licensor is obligated to retain any information provided to it in confidence only as required pursuant to the terms of the applicable Existing In-License Agreement; (d) to the extent mutually agreed to in writing by the Parties; (e) to a patent authority in connection with Prosecution and Maintenance, Defense Proceedings and enforcement of Patent Rights in accordance with ARTICLE 10; and (f) in the case of Neurocrine as Receiving Party, in Regulatory Filings for Products and, in each case under appropriate written confidentiality and non-use obligations substantially equivalent to those of this Agreement, to Third-Party contractors in connection with its Development, Manufacture and Commercialization of Collaboration Candidates and Products. The confidentiality and non-use obligations set forth under this Agreement shall survive the termination or expiration of this Agreement for a period of [**].

11.3 Press Release; Disclosure of Agreement.

11.3.1 On or promptly after the Execution Date, the Parties shall jointly issue a public announcement of the execution of this Agreement. Subject to Sections 11.3.2, 11.3.3 and 11.4, neither Party may issue any subsequent press release or other public disclosure regarding this Agreement or its terms or the Parties' activities hereunder, or any results or data arising hereunder, except: (a) with the other Party's prior written consent; (b) for any disclosure that is reasonably necessary to comply with applicable securities exchange listing requirements or other applicable Laws, provided that the Party making such disclosure provides the other Party a copy of the proposed disclosure as soon as reasonably practicable and reasonably considers any comments thereto provided by the other Party within [**] after the receipt of such proposed disclosure or such shorter period required to comply with applicable Laws; (c) to announce in a joint press release approved by both Parties Voyager's exercise of the Co-Co Option, or (d) in the case of Neurocrine, disclosure of any information relating to the Development, Manufacture or Commercialization of any Collaboration Candidate or Product that does not include Confidential Information of Voyager, provided that Neurocrine first provides Voyager a copy of the proposed disclosure and reasonably considers any timely comments thereto provided by Voyager. Notwithstanding the foregoing, to the extent information regarding this Agreement has already been publicly disclosed, each Party (other than a Party that had caused such information to become publicly disclosed in breach of this ARTICLE 11, if applicable) may subsequently disclose the same information to the public without the consent of the other Party, as long as it remains accurate at the time of subsequent disclosure.

11.3.2 Notwithstanding Section 11.3.1, each Party shall be permitted to disclose the existence and terms of this Agreement to the extent required to comply with applicable Laws or legal process, including the rules or regulations of the U.S. Securities and Exchange Commission, or similar agency in any country other than the United States, or of any stock exchange, including Nasdaq. Notwithstanding the foregoing, before disclosing this Agreement or any of the terms hereof, the Parties will coordinate in advance with each other in connection with

the redaction of certain provisions of this Agreement with respect to any filings with the U.S. Securities and Exchange Commission or similar agency in any country other than the United States, or of any stock exchange, including Nasdaq, on which securities issued by a Party or a Party's Affiliate are traded (the "Redacted Version"), and each Party will use commercially reasonable efforts to seek confidential treatment for such terms as may be reasonably requested by the other Party; provided that the Parties will use commercially reasonable efforts to file redacted versions with any governing bodies that are consistent with the Redacted Version.

11.3.3 Each Party shall be permitted to disclose the terms of this Agreement, in each case under appropriate confidentiality obligations substantially equivalent to those of this Agreement (except that the term of the obligations may be shorter as consistent with the applicable Party's ordinary business practices with regard to the protection of its confidential information), to any existing or bona fide prospective investors, lenders, professional advisors, acquirers, merger partners, licensees or Inbound Licensors, except that, with respect to any disclosure to an Inbound Licensor under an Existing In-License Agreement, Neurocrine acknowledges that the relevant Inbound Licensor is obligated to retain any information provided to it in confidence only as required pursuant to the terms of the applicable Existing In-License Agreement.

11.4 Publications. The Parties recognize that it may be useful or required to publish or publicly disclose the results of Exploitation activities conducted hereunder, and each Party will comply with the publication plan approved by the JSC for the disclosure of such results. Each Party (and its Affiliates and Sublicensees) shall be free to publish or publicly disclose such results, including on its clinical trials registry or on a government-sponsored database such as www.clinicaltrials.gov, subject to the prior review by the other Party for patentability and protection of its Confidential Information as described in this Section 11.4; provided that Voyager shall not publish or make any public announcement regarding a Collaboration Candidate or Product or any data or results generated under this Agreement relating to a Program (unless related solely to a Program Capsid), Program Target or a Program Payload without approval by the Publication Working Group. During the Term, the Party that desires to publish material requiring the review or consent of the other Party shall provide the other Party for review and approval a copy of such proposed abstract, manuscript, or presentation no less than [**] in the case of abstracts) prior to its intended submission for publication. The reviewing Party shall respond in writing promptly and in no event later than [**] in the case of abstracts) after receipt of the proposed material, with one or more of the following: (x) comments on the proposed material, which the publishing Party shall consider in good faith; (y) a specific statement of concern, based upon the need to seek patent protection or to block publication if the reviewing Party determines that the proposed disclosure contains or describes intellectual property that should be maintained as a trade secret to protect a Collaboration Candidate, Product or any Exploitation activities conducted under this Agreement; or (z) an identification of the reviewing Party's Confidential Information that is contained in the material reviewed. In the event of concern over patent protection or whether maintaining a trade secret would be a priority, the publishing Party agrees not to submit such publication or to make such presentation that contains such information until the reviewing Party is given a reasonable period of time, and in no event more than [**], unless otherwise agreed by the Parties, to seek patent protection for any material in such publication or presentation which it believes is patentable or to resolve any other issues; provided, however, that the publishing Party shall abandon such proposed publication or presentation if the reviewing Party

reasonably determines in good faith that maintaining such information as a trade secret is a commercially reasonable priority. Any Confidential Information of the reviewing Party shall, if requested by the reviewing Party, be removed. Furthermore, with respect to any proposed abstracts, manuscripts or summaries of presentations by Clinical Trial investigators, such materials shall be subject to review under this Section 11.4 to the extent that Neurocrine or Voyager (as the case may be) has the right to do so. Voyager shall not grant any other Third-Party any rights to publish results generated under this Agreement without approval by an appropriate Committee.

11.5 Remedies. Each Party shall be entitled to seek, in addition to any other right or remedy it may have, at Law or in equity, a temporary injunction, without the posting of any bond or other security, enjoining or restraining the other Party from any violation or threatened violation of this ARTICLE 11.

ARTICLE 12
REPRESENTATIONS AND WARRANTIES

12.1 Representations and Warranties of Both Parties. Each Party hereby represents and warrants to the other Party, as of the Execution Date and as of the Effective Date, that:

12.1.1 such Party is duly organized, validly existing and in good standing under the Laws of the jurisdiction of its incorporation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

12.1.2 such Party has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

12.1.3 this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, and binding obligation, enforceable against it in accordance with the terms hereof;

12.1.4 the execution, delivery and performance of this Agreement by such Party do not conflict with or result in a breach of any agreement or any provision thereof, or any instrument or understanding, oral or written, to which it is a party or by which it is bound, or any provision of the organizational documents of such Party, nor violate any Laws of any court, governmental body or administrative or other agency having jurisdiction over such Party;

12.1.5 no government authorization, consent, approval, license, exemption of or filing or registration with any court or governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, under any applicable Laws currently in effect, is or will be necessary for, or in connection with, the transactions contemplated by this Agreement or any other agreement or instrument executed in connection herewith, or for the performance by it of its obligations under this Agreement and such other agreements, except as may be required to obtain clearance of this Agreement under the HSR Act, to conduct Clinical Trials, to Manufacture Products, or to seek or obtain Regulatory Approvals;

12.1.6 since January 1, 2018, such Party and its Affiliates have conducted and will conduct their business in material compliance with the Foreign Corrupt Practices Act of 1977 and any other applicable anti-corruption Laws; and

12.1.7 such Party and, to its Knowledge, its and its Affiliates' Representatives have not directly or indirectly promised, offered or provided any unlawful corrupt payment, gratuity, emolument, bribe, kickback, illicit gift or hospitality or other illegal or unethical benefit to a Public Official or Entity or any other person in connection with the performance of such Party's obligations under this Agreement.

12.2 Representations, Warranties and Covenants, as applicable, of Voyager. Voyager hereby represents, warrants, and covenants to Neurocrine, as of the Execution Date and as of the Effective Date, that:

12.2.1 Voyager has the right to grant all rights and licenses it purports to grant to Neurocrine under this Agreement.

12.2.2 Voyager has not granted, and will not during the Term grant, any right or license to any Third-Party that would conflict with the rights or licenses granted to Neurocrine hereunder.

12.2.3 Schedule 12.2.3 sets forth a true and complete list, of all Voyager Patent Rights that Cover any Capsid that may be a Voyager Capsid, indicating the assignee(s) of each such Patent Right; and Voyager is the sole and exclusive owner of, or otherwise Controls via an exclusive license such Voyager Patent Rights, free and clear of any claims, liens, charges or encumbrances other than the Existing In-Licenses and licenses granted by Voyager that do not conflict with the licenses granted to Neurocrine under this Agreement.

12.2.4 The Inventions claimed by the Voyager Patent Rights: (a) were not conceived, discovered, developed or otherwise made in connection with any research activities funded, in whole or in part, by the federal government of the United States or any agency thereof; (b) are not a "subject invention" as that term is described in 35 U.S.C. Section 201(c); and (c) are not otherwise subject to 35 U.S.C. §§ 200-212, as amended, as well as any regulations promulgated thereunder.

12.2.5 Except as disclosed in Schedule 12.2.5, no claim or litigation has been brought or threatened in writing against Voyager or, to its Knowledge, any Third-Party by any Person alleging that the Voyager IP is infringing or, if practiced or commercialized, will infringe the rights of any Third-Party, or that the development of the Voyager IP infringed or misappropriated the intellectual property rights of any Third-Party, and to Voyager's Knowledge there is no basis for any such claim.

12.2.6 Except as disclosed in Schedule 12.2.5, to Voyager's Knowledge, the conduct of the Development Plans will not infringe any Patent Rights or misappropriate any materials, Know-How or other intellectual property of any Third Party;

12.2.7 Except as disclosed in Schedule 12.2.5, there are no judgments, orders, decrees or settlements against or owed by Voyager or any of its Affiliates, and, there is no written claim, written demand, suit, proceeding, arbitration, and to Voyager's Knowledge, other claim, demand, inquiry, investigation or other legal action of any nature, civil, criminal, regulatory or otherwise, pending or, to the Knowledge of Voyager, threatened against Voyager or any of its Affiliates, in each case relating to the Voyager IP, the Programs and Collaboration Candidates or the transactions contemplated by this Agreement.

12.2.8 To Voyager's Knowledge, no Person is infringing or threatening to infringe, or misappropriating or threatening to misappropriate, the Voyager IP, and no Person has challenged or threatened to challenge the inventorship, ownership, Voyager's right to use, scope, validity or enforceability of, or Voyager's or any Inbound Licensor's rights in or to, any Voyager Patent Rights (including through the institution or threat of institution of interference, derivation, post-grant review, opposition, nullity or similar invalidity proceedings before the United States Patent and Trademark Office or any analogous Governmental Authority).

12.2.9 To Voyager's Knowledge, the Voyager Patent Rights are valid and enforceable, or in the case of pending patent applications, will be valid and enforceable upon issuance, the inventorship of each Voyager Patent Right is properly identified on each patent and patent application, and Voyager has complied (and, to its Knowledge, its Inbound Licensors have complied) with, all applicable Laws and duties of candor with respect to the filing, prosecution and maintenance of the Voyager Patent Rights. Voyager has paid, with respect to all Voyager Patent Rights to which Voyager has prosecution and maintenance rights, and, to Voyager's Knowledge, its Inbound Licensors have paid all maintenance and annuity fees with respect to the Voyager Patent Rights due as of the Execution Date.

12.2.10 All of its Representatives have executed agreements or have existing obligations under applicable Laws requiring assignment to Voyager of all Inventions made during the course of and as the result of their association with Voyager and obligating the individual to maintain as confidential Voyager's Confidential Information as well as confidential information of other Persons (including Neurocrine and its Affiliates) which such individual may receive, in each case to the extent required to support Voyager's obligations under this Agreement.

12.2.11 (i) Neither Voyager nor, to Voyager's Knowledge, any Third-Party, is in breach of any In-License Agreement in any material respect and, to Voyager's Knowledge, each Existing In-License Agreement is in full force and effect, and neither Voyager nor any of its Affiliates has received any written notice of breach of any Existing In-License Agreements; (ii) there are no agreements between Voyager (or any of its Affiliates), on the one hand, and a Third-Party, on the other hand, pursuant to which Voyager or any of its Affiliates has Control of any Voyager IP as of the Execution Date other than those listed on Schedule 1.69, (iii) none of the Existing In-License Agreements include any obligations that restrict or conflict with the practice of the licenses granted by Neurocrine hereunder; and (iv) true, correct and complete copies of each Existing In-License Agreement have been provided to Neurocrine.

12.2.12 Except for any contract granting only a non-exclusive license to (a) a Third-Party to provide services or products to Voyager in a fee-for-service arrangement that does not convey to any Third-Party or allow any Third-Party to retain any rights in any Voyager Patent

Rights or Voyager Know-How or (b) Inbound Licensors for non-commercial research and educational purposes, there are no agreements pursuant to which Voyager or any of its Affiliates has granted any right or license to practice any Voyager Patent Rights or Voyager Know-How that would be inconsistent or in conflict with the rights granted pursuant to this Agreement.

12.2.13 Voyager has taken reasonable precautions to preserve the confidentiality of the Voyager Know-How, including requiring each Person having access to the Voyager Know-How to be subject to confidentiality, non-use and non-disclosure obligations protecting the Voyager Know-How as the confidential, proprietary materials and information of Voyager.

12.2.14 Voyager has made available to Neurocrine (a) all information in Voyager's or its Affiliates' Control related to the safety or efficacy of any Capsid that is reasonably anticipated as of the Execution Date to be a Voyager Capsid and (b) all other information in Voyager's Control requested by Neurocrine.

12.2.15 Voyager and its Affiliates have conducted, and, to Voyager's Knowledge, its and their respective contractors and consultants have conducted, all Development of Capsids reasonably anticipated as of the Execution Date to be Voyager Capsids in accordance with, as applicable, GLP, GCP and all other applicable Laws.

12.2.16 Neither Voyager nor any of its Affiliates, nor, to Voyager's Knowledge, any of its or their respective Representatives, has committed an act, made a statement or failed to act or make a statement that: (a) would be or create an untrue statement of material fact or fraudulent statement to the FDA or any other Governmental Authority with respect to the Exploitation of Capsids that may be Voyager Capsids, or any product containing any such Capsid; or (b) could reasonably be expected to provide a basis for the FDA to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery and Illegal Gratuities", set forth in 56 Fed. Reg. 46191 (September 10, 1991) and any amendments thereto or any analogous laws or policies in the Territory.

12.2.17 Voyager: (a) will promptly notify Neurocrine of any lawsuits, claims, administrative actions, regulatory inquiries or investigations, or other proceedings asserted or commenced against Voyager or its Affiliates or their respective Representatives involving in any material way the ability of Voyager to deliver the rights, licenses and sublicenses granted herein; and (b) will promptly notify Neurocrine in writing of any facts or circumstances that come to Voyager's attention and that cause, or are reasonably expected to cause, any of the representations and warranties contained in Section 12.1 or 12.2 to be untrue in any material respect at any time during the Term.

12.3 Mutual Covenants. Each Party hereby covenants to the other Party that:.

12.3.1 Such Party and its and its Affiliates' Representatives shall not, in connection with the performance of their respective obligations under this Agreement, directly or indirectly through Third-Parties, unlawfully pay, promise or offer to pay, or authorize the payment of, any money or give any promise or offer to give, or authorize the giving of anything of value to a Public

Official or Entity or other person for the purpose of corruptly obtaining or retaining business for or with, or directing business to, any person, including either Party.

12.3.2 Such Party and its Affiliates, and their Representatives, in connection with the performance of their respective obligations under this Agreement, shall not cause the other Party or its respective Affiliates, and their Representatives to be in violation of the FCPA or any other applicable Law.

12.3.3 Such Party shall without unreasonable delay notify the other Party if the notifying Party has any credible information or reasonable suspicion that there may be a violation of the FCPA or any other applicable Law in connection with the performance of this Agreement or the Development, Manufacture or Commercialization of any Program Capsid, Collaboration Candidate or Product.

12.3.4 In connection with the performance of its obligations under this Agreement, such Party shall comply and shall cause its Affiliates and their Representatives to comply with such Party's own anti-corruption and anti-bribery policy, a copy of which will be provided to the other Party within [**] of the Effective Date.

12.3.5 Either Party will have the right, upon reasonable prior written notice and during the other Party's regular business hours, to engage an independent Third-Party to audit such Party's books and records in the event that a suspected violation of any of the representations, warranties or covenants in Sections 12.3.1 through 12.3.4 needs to be investigated.

12.3.6 In the event that a Party has violated or been reasonably suspected of violating any of the covenants in Sections 12.3.1 through 12.3.4, such Party will cause its or its Affiliates' personnel or others working under its direction or control to submit to periodic training that such Party will provide on anti-corruption law compliance.

12.3.7 Either Party will, at the other Party's request, [**] certify to the other Party in writing certifying such Party's compliance, in connection with the performance of the certifying Party's obligations under this Agreement, with the covenants in Sections 12.3.1 through 12.3.4.

12.3.8 Either Party shall have the right to suspend or terminate this Agreement in its entirety where there is a credible finding, after a reasonable investigation, that the other Party, in connection with performance of its obligations under this Agreement, has violated the FCPA.

12.3.9 All individuals who are employees or independent contractors of such Party or any of its Affiliates working under this Agreement are and will be under written obligation to assign or, in the case of independent contractors, assign or exclusively license, all right, title and interest in and to all Inventions and other Know-How, and all intellectual property rights therein, developed under this Agreement to such Party or its Affiliate as the sole owner or exclusive licensee thereof.

12.3.10 Such Party will not employ, or use any contractor or consultant that employs or uses, any Person: (a) that is debarred by the FDA (or subject to a similar sanction of EMA or any other Governmental Authority); or (b) to such Party's Knowledge, that is the subject

of an FDA debarment investigation or proceeding (or similar proceeding of EMA or any other Governmental Authority); in each of clauses (a) and (b) in the conduct of its activities under this Agreement;

12.3.11 In performing its obligations or exercising its rights under this Agreement, such Party, its Affiliates, and, with respect to Neurocrine, its Sublicensees, shall comply in all material respects with all applicable Law, including all anti-corruption Laws; and

12.3.12 Such Party will not grant any license relating to the Voyager IP (if such Party is Voyager) or the Neurocrine IP (if such Party is Neurocrine) that would conflict with the rights or licenses granted or to be granted to the other Party hereunder.

12.4 Disclaimer. Except as otherwise expressly set forth in this Agreement, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ANY WARRANTY THAT ANY PATENTS ARE VALID OR ENFORCEABLE, AND EXPRESSLY DISCLAIMS ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NONINFRINGEMENT.

ARTICLE 13
INDEMNIFICATION; INSURANCE

13.1 Indemnification by Neurocrine. Subject to Section 13.3 and the terms of the Co-Co Agreement, Neurocrine shall indemnify, hold harmless and defend Voyager and its Affiliates, and its or their respective Representatives, from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys and other professional advisors (collectively, "Losses"), to the extent arising out of or resulting from any Third-Party suits, claims, actions, proceedings or demands ("Third-Party Claims") to the extent resulting from:

13.1.1 the gross negligence, recklessness or intentional misconduct of Neurocrine or any of its Affiliates, or its or their respective Representatives, in connection with performance by or on behalf of Neurocrine of Neurocrine's obligations or exercise of Neurocrine's rights under this Agreement;

13.1.2 any breach of this Agreement, including any representation or warranty or covenant, by Neurocrine; or

13.1.3 the Exploitation of Collaboration Candidates or Products conducted by or on behalf of Neurocrine (it being agreed that any activities by or on behalf of Voyager under this Agreement will not be considered "on behalf of Neurocrine" for purposes of this Section 13.1.3), any of its Affiliates or any Sublicensee, including: (a) any product liability, personal injury, property damage or other damage; and (b) infringement of any Patent Rights or other intellectual property rights of any Third-Party, except to the extent related to any Voyager Capsid or any Capsid IP licensed to Neurocrine hereunder; provided, however, that Losses arising from Exploitation of any Product under any Co-Co Agreement shall be shared as Development Costs or profit or loss, as applicable, in accordance with the term of such Co-Co Agreement;

except, in each case (13.1.1 through 13.1.3), to the extent arising from the gross negligence, recklessness or intentional misconduct of Voyager or any of its Affiliates or its or their respective Representatives or Voyager's breach of this Agreement, including any representation, warranty or covenant.

13.2 Indemnification by Voyager. Subject to Section 13.3 and the terms of the Co-Co Agreement, Voyager shall indemnify, hold harmless and defend, Neurocrine and its Affiliates, and its or their respective Representatives, from and against any and all Losses, to the extent arising out of or resulting from any Third-Party Claims to the extent resulting from:

13.2.1 the gross negligence, recklessness or intentional misconduct of Voyager or any of its Affiliates or subcontractors, or its or their respective Representatives, in connection with performance by or on behalf of Voyager of Voyager's obligations or exercise of Voyager's rights under this Agreement;

13.2.2 any breach of this Agreement, including any representation or warranty or covenant, by Voyager; or

13.2.3 the Exploitation of Collaboration Candidates or Products conducted by or on behalf of Voyager or any of its Affiliates, or any of their respective licensees (excluding Neurocrine), before the Effective Date or after termination of this Agreement, including: (a) any product liability, personal injury, property damage or other damage; and (b) infringement of any Patent Rights or other intellectual property rights of any Third-Party; provided, however, that Losses arising from Exploitation of any Product under any Co-Co Agreement shall be shared as Development Costs or profit or loss, as applicable, in accordance with the term of such Co-Co Agreement;

except, in each case (13.2.1 through 13.2.3), to the extent arising from the gross negligence, recklessness or intentional misconduct of Neurocrine or any of its Affiliates or its or their respective Representatives or Neurocrine's breach of this Agreement, including any representation, warranty or covenant.

13.3 Procedure. A Person entitled to indemnification under this ARTICLE 13 (an "Indemnified Party") shall give prompt written notification to the Person from whom indemnification is sought (the "Indemnifying Party") of the commencement of any Third-Party Claim for which indemnification may be sought or, if earlier, upon the assertion of any such Third-Party Claim (it being understood and agreed, however, that the failure by an Indemnified Party to give notice of a Third-Party Claim as provided in this Section 13.3 shall not relieve the Indemnifying Party of its indemnification obligation under this Agreement except and only to the extent that such Indemnifying Party is actually damaged as a result of such failure to give notice). Within [**] after delivery of such notification, the Indemnifying Party may, upon written notice thereof to the Indemnified Party, assume control of the defense of such Third-Party Claim with counsel reasonably satisfactory to the Indemnified Party. If the Indemnifying Party does not assume control of such defense, the Indemnified Party shall control such defense and, without limiting the Indemnifying Party's indemnification obligations, the Indemnifying Party shall reimburse the Indemnified Party for all reasonable costs and expenses, including attorney fees, incurred by the Indemnified Party in defending itself within [**] after receipt of any reasonably

detailed invoice and supporting documentation therefor from the Indemnified Party. The Party not controlling such defense may participate therein at its own expense; provided that, if the Indemnifying Party assumes control of such defense and the Indemnified Party in good faith concludes, based on advice from counsel, that the Indemnifying Party and the Indemnified Party have conflicting interests with respect to such Third-Party Claim, the Indemnifying Party shall be responsible for the reasonable fees and expenses of counsel to the Indemnified Party in connection therewith. The Party controlling such defense shall keep the other Party advised of the status of such Third-Party Claim and the defense thereof and shall consider recommendations made by the other Party with respect thereto. The Indemnified Party shall not agree to any settlement of such Third-Party Claim without the prior written consent of the Indemnifying Party, which shall not be unreasonably withheld, delayed or conditioned. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party, which shall not be unreasonably withheld, delayed or conditioned, agree to any settlement of such Third-Party Claim or consent to any judgment in respect thereof that does not include a complete and unconditional release of the Indemnified Party from all liability with respect thereto, that imposes any liability or obligation on the Indemnified Party or that acknowledges fault by the Indemnified Party.

13.4 Insurance. Subject to the terms of any Co-Co Agreement:

13.4.1 Voyager's Insurance Obligations. Voyager shall maintain, at its cost, insurance against liability and other risks associated with its activities and obligations under this Agreement, including its Clinical Trials and its indemnification obligations hereunder, in such amounts, subject to such deductibles and on such terms as are reasonable for a company such as Voyager for the activities to be conducted by it under this Agreement. Voyager shall furnish to Neurocrine evidence of such insurance upon request.

13.4.2 Neurocrine's Insurance Obligations. Neurocrine shall maintain, at its cost, insurance against liability and other risks associated with its and its Affiliates' and any Sublicensees' activities and obligations under this Agreement, including Clinical Trials, the Exploitation of Collaboration Candidates and Products and Neurocrine's indemnification obligations hereunder, in such amounts and on such terms as are reasonable and customary for a company such as Neurocrine for the activities to be conducted by it under this Agreement. Neurocrine shall furnish to Voyager evidence of such insurance upon request.

13.5 Limitation of Liability. EXCEPT FOR A BREACH OF ARTICLE 9 OR ARTICLE 11 OR FOR CLAIMS OF A THIRD-PARTY THAT ARE SUBJECT TO INDEMNIFICATION UNDER THIS ARTICLE 13, NEITHER VOYAGER NOR NEUROCRINE, NOR ANY OF THEIR RESPECTIVE AFFILIATES, LICENSORS, LICENSEES OR SUBLICENSEES, WILL BE LIABLE TO THE OTHER PARTY, ITS AFFILIATES OR SUBLICENSEES FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL OR PUNITIVE DAMAGES, OR LOST PROFITS, ROYALTIES, DATA OR PROCUREMENT OF SUBSTITUTE GOODS, WHETHER LIABILITY IS ASSERTED IN CONTRACT, TORT (INCLUDING NEGLIGENCE AND STRICT PRODUCT LIABILITY), INDEMNITY OR CONTRIBUTION, AND IRRESPECTIVE OF WHETHER THAT PARTY OR ANY REPRESENTATIVE OF THAT PARTY HAS BEEN ADVISED OF, OR OTHERWISE MIGHT HAVE ANTICIPATED THE POSSIBILITY OF, ANY SUCH LOSS OR DAMAGE.

ARTICLE 14
TERM AND TERMINATION

14.1 Term. This Agreement shall commence as of the Effective Date and, unless terminated earlier, this Agreement shall continue in full force and effect until the later of: (a) the expiration of the last to expire Royalty Term with respect to all Products in all countries in the Territory; or (b) the expiration or termination of any Co-Co Agreement (the "Term").

14.2 Termination by Neurocrine.

14.2.1 Neurocrine may terminate this Agreement in its entirety or on a Program-by-Program and/or country-by-country basis by providing written notice of termination to Voyager, which notice specifies the scope of the termination and includes an effective date of termination at least: (a) one hundred eighty (180) days after the date of the notice if such notice is provided prior to First Commercial Sale of any Product to which the termination applies; or (b) one (1) year after the date of the notice if such notice is provided after First Commercial Sale of any Product to which the termination applies.

14.2.2 Neurocrine may terminate this Agreement with respect to a given Product by providing written notice of termination to Voyager within thirty (30) days after complete readout of any Clinical Trial if: (a) the results of such Clinical Trial fail to meet the pre-specified primary endpoint(s) set forth in the protocol therefor; or (b) a Significant Safety Signal occurred during such Clinical Trial.

14.3 Termination for Breach.

14.3.1 This Agreement may be terminated: (a) on a Program-by-Program basis, at any time upon written notice by either Party if the other Party is in material breach of this Agreement with respect to such Program; or (b) in its entirety, at any time upon written notice by either Party if the other Party is in material breach of this Agreement with respect to all Programs, or if such material breach does not relate specifically to any Program; in either case ((a) or (b)) except if the breaching Party has cured such breach within [**] in the case of a payment breach ([**] in the case of the Initial Fee), or within [**] in the case of all other breaches, after the non-breaching Party has provided written notice to the breaching Party of such breach; provided that if the breach is curable but is not capable of cure within such [**] period, then the cure period will be extended for so long as the breaching Party is diligently implementing a cure plan reasonably designed to cure such breach, provided that, such cure period does not exceed [**] in total.

14.3.2 Without limiting Section 14.3.1, if the applicable material breach is a material breach by Neurocrine of its obligations under Section 4.2.2 to use Commercially Reasonable Efforts with respect to a Program in one or more, but not all, of the Major Market Countries, then Voyager will not have the right to terminate this Agreement with respect to such Program in all countries but instead may terminate this Agreement with respect to such Program only in the Major Market Country(ies) in which there was an uncured material breach by Neurocrine with respect to its obligation to use Commercially Reasonable Efforts.

14.3.3 If the Parties reasonably and in good faith disagree as to whether there has been a material breach, the Party that seeks to dispute that there has been a material breach shall contest the allegation in accordance with Section 15.2 during the applicable cure period. The cure period for any allegation made in good faith as to a material breach under this Agreement will, subject to Sections 14.3.1 and 15.3, including the suspension of such cure period set forth therein, run from the date that written notice of breach was first provided to the breaching Party by the non-breaching Party.

14.4 Termination for Failure to Make Equity Purchase. If Neurocrine fails to purchase from Voyager shares of Voyager common stock pursuant to the terms and within the timeframe specified in the Stock Purchase Agreement (subject to any cure provisions therein), then Voyager shall have the right to terminate this Agreement in its entirety upon written notice to Neurocrine.

14.5 Termination for Patent Challenge. If, during the Term, Neurocrine (a) commences or participates in any action or proceeding (including any patent opposition or re-examination proceeding), or otherwise asserts any claim, challenging or denying the validity or enforceability of any claim of Voyager Patent Rights, except in the normal course of patent prosecution, or (b) actively assists any other Person in bringing or prosecuting any action or proceeding (including any patent opposition or reexamination proceeding) challenging or denying the validity or enforceability of any claim of Voyager Patent Rights (each of (a) and (b), a "Patent Challenge"), then, to the extent permitted by applicable Laws, Voyager shall have the right, exercisable within [**] following receipt of notice regarding such Patent Challenge, in its sole discretion, to terminate this Agreement with respect to such Voyager Patent Right(s), such termination to be effective [**] following such notice (or such longer period as Voyager may designate in such notice) unless Neurocrine withdraws or causes to be withdrawn all such challenge(s) (or in the case of *ex-parte* proceedings, multi-party proceedings, or other Patent Challenges that Neurocrine does not have the power to unilaterally withdraw or cause to be withdrawn, Neurocrine ceases actively assisting any other party to such Patent Challenge and, to the extent Neurocrine is a party to such Patent Challenge, it withdraws from such Patent Challenge) within such [**] period. The foregoing sentence shall not apply (i) with respect to any Voyager Patent Rights that Voyager first asserts against Neurocrine or any of its Affiliates where the Patent Challenge is made in defense of such assertion, or (ii) with respect to any Patent Challenge commenced by a Third-Party that after the Execution Date acquires or is acquired by Neurocrine or its Affiliates or its or their business or assets, whether by stock purchase, merger, asset purchase or otherwise, but only with respect to Patent Challenges commenced prior to the closing of such acquisition. The following will not be considered a Patent Challenge: (A) responding to compulsory discovery, subpoenas or other requests for information in a judicial or arbitration proceeding; or (B) complying with any applicable Law or court order.

14.6 Effects of Termination other than by Neurocrine for Voyager Breach. Without limiting any other legal or equitable remedies that either Party may have, if this Agreement is terminated (in whole or in part) for any reason except by Neurocrine pursuant to Section 14.3 above, then upon effectiveness of such termination the following shall apply, provided that if termination of this Agreement is limited to a particular country(ies), Product(s) or Program(s) then the following shall apply only with respect to such country(ies), Product(s) or Program(s):

14.6.1 the license grants to Neurocrine in Section 15.1 shall terminate immediately on the effective date of termination;

14.6.2 Neurocrine shall, and hereby does, effective upon such termination, grant to Voyager a royalty-bearing, sublicenseable (through multiple tiers) license under the Neurocrine IP that is necessary for the use of any Terminated Products or is otherwise incorporated into Terminated Products as of the effective date of termination, to Exploit Terminated Products in the terminated country(ies), which license will be non-exclusive or exclusive as requested by Voyager; the Parties shall negotiate in good faith commercially reasonable royalties payable by Voyager to Neurocrine on sales of such Terminated Products, which shall reflect the value of, and Neurocrine's investment in the development of, such Terminated Products and the exclusivity of the license, and the terms related to such royalty payments;

14.6.3 if Voyager so requests, and to the extent permitted under the relevant agreement at the time of termination, Neurocrine shall transfer to Voyager any agreements between Neurocrine or any of its Affiliates, on the one hand, and any Affiliate or Third-Party, on the other hand, to the extent relating to the Exploitation of any Terminated Product in the terminated country(ies) to which Neurocrine or any of its Affiliates or any Sublicensees is a party, subject to any required consents of such Third-Party, which Neurocrine shall use commercially reasonable efforts to obtain promptly (but shall not be obligated to pay any additional consideration to such Third-Party);

14.6.4 if Voyager so requests, Neurocrine shall transfer all right, title and interest to any Regulatory Filings (including all Regulatory Approvals), pricing and reimbursement approvals in the terminated country(ies) with respect to the Terminated Products to Voyager;

14.6.5 Neurocrine shall provide any other assistance reasonably requested by Voyager for the purpose of allowing Voyager or its designee to proceed expeditiously with the Exploitation of Terminated Products in the Field in the terminated country(ies) following Voyager's receipt of the written notice of termination and over a [**] period following the effective date of the termination, and Voyager shall pay Neurocrine's FTE Costs and Out-of-Pocket Costs to conduct such assistance (except in the event Voyager terminated this Agreement pursuant to Section 14.3 above);

14.6.6 Neurocrine shall, and shall cause its Affiliates and shall use Commercially Reasonable Efforts to cause its Sublicensees to, execute all documents as may be reasonably requested by Voyager in order to give effect to the foregoing clauses; and

14.6.7 if this Agreement is terminated in its entirety, each Party shall return to the other Party any Confidential Information of the other Party, or shall destroy, and certify the destruction in writing any Confidential Information of the other Party, except for any such Confidential Information that Voyager has the right to use pursuant to the terms of this Agreement.

14.7 Effects of Termination by Neurocrine for Voyager Breach. If Neurocrine terminates this Agreement with respect to one or more Programs pursuant to Section 14.3, then all rights and obligations under this Agreement with respect to such Terminated Programs will terminate, except as expressly provided in Section 14.9, and if such termination is of this Agreement in its entirety,

Voyager shall return to Neurocrine or destroy, and certify such destruction in writing, any Confidential Information of Neurocrine. If Neurocrine has the right to terminate this Agreement with respect to one or more Programs for Voyager's material breach pursuant to Section 14.3, then in lieu of termination, and in addition to the remedies provided in Section 2.1.6, Neurocrine shall have the right to keep this Agreement in effect and to elect one or both of the following remedies upon written notice to Voyager:

14.7.1 if such Programs include the GBA1 Program, then the Co-Co Option will terminate, and if a Co-Co Agreement is then in effect with respect to the GBA1 Program, then such Co-Co Agreement will terminate, and Voyager will no longer have the right to co-develop and co-commercialize the applicable Products with Neurocrine; and

14.7.2 subject to the applicable terms of any In-License Agreement, Neurocrine shall no longer have any obligations with respect to diligence or to use Commercially Reasonable Efforts with respect to any Products resulting from the applicable Programs.

14.8 HSR Filing; Termination.

14.8.1 Except for the Parties' obligations under ARTICLE 11, ARTICLE 12 and this Section 14.8, which shall be effective as of the Execution Date, this Agreement shall not become effective until the Effective Date.

14.8.2 Each Party will use reasonable efforts to do, or cause to be done, all things necessary, proper and advisable to, as promptly as practicable, take all actions necessary to obtain expiration or termination of all applicable waiting periods and requests for information (and any extensions thereof) under the HSR Act, including filing with the U.S. Federal Trade Commission and the Antitrust Division of the U.S. Department of Justice, any HSR Filing required of it under the HSR Act with respect to the transactions contemplated hereby within thirty (30) days after the Execution Date (or such later time as may be agreed to in writing by the Parties). The Parties shall cooperate with one another to the extent necessary in the preparation of any such HSR Filing and during the review by the U.S. Federal Trade Commission or the Antitrust Division of the U.S. Department of Justice. Each Party shall be responsible for its own costs and expenses; provided, however, that Neurocrine shall be solely responsible for any fees (other than penalties that may be incurred as a result of actions or omissions on the part of Voyager) required to be paid in connection with making any such HSR Filing. If the Parties make an HSR Filing hereunder, then this Agreement shall terminate: (a) at the election of either Party, immediately upon notice to the other Party, if the U.S. Federal Trade Commission or the U.S. Department of Justice seeks a preliminary injunction under the Antitrust Laws to enjoin the transactions contemplated by this Agreement or the U.S. Federal Trade Commission issues a complaint pursuant to Section 5(b) of the FTC Act; or (b) at the election of either Party, immediately upon notice to the other Party, in the event that the HSR Clearance Date shall not have occurred on or prior to twelve (12) months after the effective date of the HSR Filing. In the event of such termination, this Agreement shall be of no further force and effect.

14.9 Accrued Rights; Surviving Provisions of the Agreement.

14.9.1 Termination or expiration of this Agreement for any reason shall be without prejudice to any rights that shall have accrued to the benefit of any Party prior to such termination or expiration, including any payment obligations hereunder, and any and all damages or remedies arising from any breach hereunder. Such termination or expiration shall not relieve any Party from obligations which are expressly indicated to survive expiration or termination of this Agreement.

14.9.2 The provisions of Sections 2.2, 4.1.3, 5.2.4 and 7.3 (but in each case, with respect to the payment obligations therein, solely to the extent such payment obligations have accrued as of the date of termination); Sections 7.4, 10.1.1 through 10.1.3, 10.4, 12.4, 14.6, 14.7 and 14.9; and ARTICLE 1 (for the purpose of interpreting this Agreement), ARTICLE 8 (but with respect to the payment obligations therein, solely to the extent payable as of the date of termination), ARTICLE 11 (excluding 11.4 to the extent related publication of information related to any Terminated Program that is not the other Party's Confidential Information), ARTICLE 13 (excluding Section 13.4) and ARTICLE 15, shall survive the termination of this Agreement in its entirety or expiration of this Agreement for any reason, in accordance with their respective terms and conditions, and for the duration stated, and where no duration is stated, shall survive indefinitely.

ARTICLE 15
MISCELLANEOUS

15.1 Governing Law. This Agreement and any dispute arising from the performance or breach hereof shall be governed by and construed in accordance with the Laws of the State of New York without reference to conflicts of laws principles; provided that with respect to matters involving the enforcement of intellectual property rights, the Laws of the applicable country shall apply. The provisions of the United Nations Convention on Contracts for the International Sale of Goods shall not apply to this Agreement or any subject matter hereof.

15.2 Dispute Resolution. Except for the disputes at the JSC, which matters shall be resolved as provided in Section 3.6, in the event of any dispute arising out of or in connection with this Agreement ("Dispute"), either Party shall refer such Dispute in writing to the Parties' respective Executive Officers, and such Executive Officers shall attempt in good faith to resolve such Dispute. If the Dispute is not resolved within [**] after it has been referred to the Executive Officers, the Dispute shall be finally settled through binding arbitration pursuant to Section 15.3. Any disputes concerning the propriety of the commencement of arbitration shall be finally settled by the arbitral tribunal.

15.3 Arbitration Request.

15.3.1 No Arbitration of Patent Issues. Any dispute, controversy or claim relating to the scope, validity, enforceability or infringement of any Patents Covering the use, importation, Manufacture, offer for sale or sale of Products shall be submitted to a court of competent jurisdiction in the country in which such Patents were granted or arose.

15.3.2 Arbitration Procedure. Any other Disputes that have not been amicably resolved pursuant to Section 15.2 shall be finally settled under the Commercial Arbitration Rules of the American Arbitration Association (the "AAA") before a tribunal composed of three

arbitrators appointed in compliance with such rules, except as modified by this Section 15.2. Each Party shall nominate one arbitrator and within [**] of the second arbitrator's appointment, the two party-nominated arbitrators shall nominate the third arbitrator, who shall serve as president of the tribunal. None of the arbitrators shall have worked for, or been a consultant to, either Party or its Affiliates within [**] prior to the arbitration. The arbitrators shall have experience in pharmaceutical licensing disputes. An arbitrator shall be deemed to meet these qualification unless a Party objects within [**] after the arbitrator is nominated. The seat, or legal place, of the arbitration will be New York City, New York, United States. The language of the arbitration shall be English. The Parties shall mutually agree on the rules to govern discovery and the rules of evidence for the arbitration within [**] after the commencement of the arbitration. If the Parties fail to timely agree to such rules, the United States Federal Rules of Civil Procedure will govern discovery and the United States Federal Rules of Evidence will govern evidence for the arbitration. Subject to Section 13.5, the arbitrators shall be authorized to award compensatory damages, but shall not be authorized to award punitive, special, consequential, or any other similar form of damages, or to reform, modify, or materially change this Agreement. The arbitrators shall also be authorized to grant temporary, preliminary or permanent equitable remedies or relief, including an injunction or order for specific performance. The award of the arbitrators shall be the sole and exclusive remedy of the Parties, except for those remedies that are set forth in this Agreement or which apply to a Party by operation of the applicable provisions of this Agreement, and the Parties hereby expressly agree to waive the right to appeal from the decisions of the arbitrator, and there shall be no appeal to any court or other authority (government or private) from the decision of the arbitrator. Judgment on the award rendered by the arbitrators may be entered in any court of competent jurisdiction.

15.3.3 Costs. During the pendency of the arbitration each Party shall bear its own attorneys' fees, costs, and expenses of the arbitration, and shall pay an equal share of the fees and costs of the arbitrators and the AAA administrative expenses; provided, however, that the arbitrators, in their final award, shall be authorized to determine whether a Party is the prevailing Party, and if so, to award to that prevailing Party its costs and expenses of arbitration, including its reasonable attorneys' fees, the fees and costs of the arbitrators and AAA, and other costs and expenses (including, for example, expert witness fees and expenses, transcripts, photocopy charges and travel expenses), as determined by the arbitrators.

15.3.4 Preliminary Injunctions. Notwithstanding anything in this Agreement to the contrary, a Party may seek a temporary restraining order, preliminary injunction or other interim relief from any court of competent jurisdiction in order to prevent immediate and irreparable injury, loss, or damage on a provisional basis, pending the award of the arbitrators on the ultimate merits of any dispute.

15.3.5 Confidentiality. The Parties agree that the arbitration shall be kept confidential. The existence and contents of the arbitration, any non-public information provided in the arbitration, and any submissions, orders or awards made in the arbitration shall be deemed Confidential Information of each of the Parties and subject to ARTICLE 11, except that a Party may disclose such information to the arbitrators, the AAA, its counsel, experts, witnesses and any other person to the extent required for the conduct of the arbitration, or as required by applicable

Law, to protect or pursue a legal right, or to enforce or challenge an award in *bona fide* legal proceedings.

15.3.6 Suspension of Cure Period. From the date the AAA receives the request for arbitration and until such time as the Dispute has been finally settled, the running of the time periods as to which Party must cure a breach of this Agreement shall be suspended as to any breach that has been referred to arbitration.

15.3.7 Consolidation. In order to facilitate the comprehensive resolution of related disputes, and upon request of any Party to the arbitration proceeding, the AAA may consolidate the arbitration proceeding with any other arbitration relating to this Agreement or to any Co-Co Agreement.

15.4 Assignment. This Agreement may not be assigned or otherwise transferred, nor may any right or obligation hereunder be assigned or transferred, by either Party without the written consent of the other Party, which consent shall not be unreasonably withheld, conditioned or delayed; provided, however, that either Party may, without the other Party's written consent, assign this Agreement and its rights and obligations hereunder in whole or in part to: (a) an Affiliate; or (b) the Acquirer in the context of a Change of Control. Any purported assignment in violation of this Section 15.4 shall be void.

15.5 Change of Control.

15.5.1 Voyager shall notify Neurocrine in writing within [**] after entering into any agreement providing for or intended to result in any Change of Control of Voyager, identifying the parties to such agreement.

15.5.2 Following the effectiveness of such Change of Control, (a) if the Acquirer is Developing or Commercializing a product that directly competes with a Product being Developed or Commercialized by Neurocrine as of the date of the Change of Control, then Neurocrine shall have the right to disband all Committees and to require Voyager to adopt reasonable procedures, to be agreed upon in writing with Neurocrine, as reasonably necessary to limit the dissemination of Neurocrine's Confidential Information to only those personnel having a need to know such Confidential Information in order for Voyager to perform its obligations or to exercise its rights under this Agreement, (b) to the extent applicable, Section 4.1.4(b) and 4.1.4(c) will apply, and (c) if the Acquirer is Developing or Commercializing a product that directly competes with a Product being Developed or Commercialized by Neurocrine, Neurocrine will have the rights set forth in Section 2.1.4 (as if Voyager had materially breached its Development obligations and failed to cure such breach).

15.5.3 Voyager covenants that, following a Change of Control of Voyager: (a) there will be no material change in the level or nature of efforts or resources expended by Voyager with respect to, or the qualifications and experience of the personnel assigned to (including with respect to the allocation of their time to), any Program; and (b) each employee of Voyager or its Affiliates who worked on any Program during the [**] period immediately prior to the Change of Control or who would reasonably be expected to work on any Program thereafter will continue to work on such Program for so long as s/he remains an employee of Voyager or any of its Affiliates.

15.6 Performance by Affiliates and Sublicensees. Each Party hereby acknowledges and agrees that it shall be responsible for the full and timely performance as and when due under, and observance of all applicable covenants, terms, conditions and agreements set forth in this Agreement by its Affiliate(s), licensees and Sublicensees.

15.7 Force Majeure. No Party shall be held liable or responsible to the other Party nor be deemed to be in default under, or in breach of any provision of, this Agreement for failure or delay in fulfilling or performing any obligation (other than a payment obligation) of this Agreement when such failure or delay is due to force majeure. For purposes of this Agreement, force majeure is defined as any cause beyond the reasonable control of the affected Party and without the fault or negligence of such Party, which may include acts of God; material changes in Law; war; civil commotion; destruction of production facilities or materials by fire, flood, earthquake, explosion or storm; labor disturbances; epidemic or pandemic; and failure of public utilities or common carriers. In such event the Party affected by such force majeure shall immediately notify the other Party of such inability and of the period for which such inability is expected to continue. The Party giving such notice shall thereupon be excused from such of its obligations under this Agreement as it is thereby disabled from performing for so long as it is so disabled for up to a maximum of [**], after which time the Parties shall promptly meet to discuss in good faith how to best proceed in a manner that maintains and abides by the Agreement. To the extent possible, each Party shall use reasonable efforts to minimize the duration of any force majeure.

15.8 Notices. Any notice or request required or permitted to be given under or in connection with this Agreement shall be deemed to have been sufficiently given if in writing and personally delivered or sent by certified mail (return receipt requested), reputable overnight express courier service (signature required), prepaid, or e-mail (with confirmed delivery) to the Party for which such notice is intended, at the address set forth for such Party below:

If to Voyager,

addressed to: Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: Chief Executive Officer

with a copy to: Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: General Counsel
E-mail: [**]

and

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street

New York, NY 10007
Attention: Brian A. Johnson, Sarah Tegan Hogan and Jenna Ventorino
Email: brian.johnson@wilmerhale.com, sarah.hogan@wilmerhale.com and jenna.ventorino@wilmerhale.com

If to Neurocrine,

addressed to: Neurocrine Biosciences, Inc.
12780 El Camino Real
San Diego, CA 92130
Attention: Chief Legal Officer
Email: [**]

with a copy to: Cooley LLP
4401 Eastgate Mall
San Diego, CA 92121
Attention: Jason L. Kent
Email: jkent@cooley.com

or to such other address for such Party as it shall have specified by like notice to the other Party, provided that notices of a change of address shall be effective only upon receipt thereof. If delivered personally, the date of delivery shall be deemed to be the date on which such notice or request was given. If sent by overnight express courier service, the date of delivery shall be deemed to be the next Business Day after such notice or request was deposited with such service. If sent by certified mail, the date of delivery shall be deemed to be the third (3rd) Business Day after such notice or request was deposited with the U.S. Postal Service.

15.9 Export Clause. Each Party acknowledges that the Laws of the United States restrict the export and re-export of commodities and technical data of United States origin. Each Party agrees that it will not export or re-export restricted commodities or the technical data of the other Party in any form without the appropriate United States and foreign government licenses.

15.10 Waiver. Neither Party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either Party to assert a right hereunder or to insist upon compliance with any term of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either Party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

15.11 Severability. If any provision hereof should be invalid, illegal or unenforceable in any jurisdiction, the Parties shall negotiate in good faith a valid, legal and enforceable substitute provision that most nearly reflects the original intent of the Parties and all other provisions hereof shall remain in full force and effect in such jurisdiction and shall be liberally construed in order to carry out the intentions of the Parties as nearly as may be possible. Such invalidity, illegality or

unenforceability shall not affect the validity, legality or enforceability of such provision in any other jurisdiction.

15.12 Entire Agreement. This Agreement, together with the Schedules hereto, sets forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the Parties and supersede and terminate all prior agreements and understanding between the Parties. In particular, and without limitation, this Agreement supersedes and replaces the Existing Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties prior to the Execution Date. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the Parties other than as set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the Parties unless reduced to writing and signed by the respective authorized officers of the Parties. The Parties acknowledge and agree that the 2019 CLA is a separate and independent agreement, and this Agreement and the 2019 CLA shall remain in effect in accordance with their terms and shall have no impact on one another.

15.13 Independent Contractors. Nothing herein shall be construed to create any relationship of employer and employee, agent and principal, partnership or joint venture between the Parties. Each Party is an independent contractor. Neither Party shall assume, either directly or indirectly, any liability of or for the other Party. Neither Party shall have the authority to bind or obligate the other Party and neither Party shall represent that it has such authority.

15.14 CREATE Act. It is the intention of the Parties that this Agreement is a "joint research agreement" as that phrase is defined in Section 35 U.S.C. 100(h). Notwithstanding anything to the contrary in this Agreement, each Party will have the right to invoke the America Invents Act Joint Research Agreement exception codified at 35 U.S.C. § 102(c) (the "JRA Exception") when exercising its rights under this Agreement, but only with prior written consent of the other Party in its sole discretion. In the event that a Party intends to invoke the JRA Exception, once agreed to by the other Party if required by the preceding sentence, it will notify the other Party and the other Party will cooperate and coordinate its activities with such Party with respect to any filings or other activities in support thereof.

15.15 Headings; Construction; Interpretation. Headings and any table of contents used herein are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. The terms of this Agreement represent the results of negotiations between the Parties and their representatives, each of which has been represented by counsel of its own choosing, and neither of which has acted under duress or compulsion, whether legal, economic or otherwise. Accordingly, the terms of this Agreement shall be interpreted and construed in accordance with their usual and customary meanings, and each of the Parties hereby waives the application in connection with the interpretation and construction of this Agreement of any rule of Law to the effect that ambiguous or conflicting terms or provisions contained in this Agreement shall be interpreted or construed against the Party whose attorney prepared the executed draft or any earlier draft of this Agreement. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause or Schedule shall be deemed to be a reference to any Article, Section, subsection, paragraph, clause or Schedule, of or to, as the case may be, this Agreement. Except where the context otherwise requires: (a) any definition of or reference to any agreement,

instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein); (b) any reference to any Law refers to such Law including all rules and regulations thereunder and any successor Law, in each case as from time to time enacted, repealed or amended; (c) the words "herein," "hereof" and "hereunder," and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof; (d) the words "include," "includes," and "including" shall be deemed to be followed by the phrase "but not limited to," "without limitation" or words of similar import, (e) the word "or" is used in the inclusive sense (and/or); (f) words in the singular or plural form include the plural and singular form, respectively; (g) references to any gender refer to each other gender; (h) references to a particular Person include such Person's successors and assigns to the extent not prohibited by this Agreement; and (i) a capitalized term not defined herein but reflecting a different part of speech than a capitalized term which is defined herein shall be interpreted in a correlative manner.

15.16 Further Actions. Each Party shall execute, acknowledge and deliver such further instruments, and do all such other acts, as may be necessary or appropriate in order to carry out the expressly stated purposes and the clear intent of this Agreement.

15.17 Parties in Interest. All of the terms and provisions of this Agreement shall be binding upon, and shall inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

15.18 Counterparts. This Agreement may be signed in counterparts, each and every one of which shall be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

[Signature page follows.]

IN WITNESS WHEREOF, and intending to be legally bound hereby, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Execution Date.

Voyager Therapeutics, Inc.

By: /s/ Alfred W. Sandrock, Jr.
Name: Alfred W. Sandrock, Jr. M.D., Ph.D.
Title: President & CEO

Neurocrine Biosciences, Inc.

By: /s/ Kevin C. Gorman
Name: Kevin C. Gorman, Ph.D.
Title: Chief Executive Officer

[*Signature page to Collaboration and License Agreement*]

EXHIBIT A

Stock Purchase Agreement

Incorporated by reference to Exhibit 10.44 of the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 filed with the Securities and Exchange Commission

Exhibit 10.46

EXECUTION VERSION

AMENDED AND RESTATED INVESTOR AGREEMENT

By and Between

NEUROCRINE BIOSCIENCES, INC.

AND

VOYAGER THERAPEUTICS, INC.

Dated as of January 8, 2023

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Exhibit A – Form of Irrevocable Proxy
Exhibit B – Notices

INVESTOR AGREEMENT

THIS AMENDED AND RESTATED INVESTOR AGREEMENT (this "**Agreement**") is made as of January 8, 2023, by and between Neurocrine Biosciences, Inc. (the "**Investor**"), a Delaware corporation with its principal place of business at 12780 El Camino Real, San Diego, CA 92130, and Voyager Therapeutics, Inc. (the "**Company**"), a Delaware corporation with its principal place of business at 64 Sidney Street, Cambridge, MA 02139.

WHEREAS, in connection with entering into that certain Collaboration and License Agreement, dated January 28, 2019, by and between the Investor and the Company (the "**Prior Collaboration Agreement**") and that certain Stock Purchase Agreement, dated January 28, 2019, by and between the Investor and the Company (the "**Prior Purchase Agreement**"), the Investor and the Company entered into that certain Investor Agreement, dated January 28, 2019, by and between the Investor and the Company (the "**Prior Investor Agreement**"), pursuant to which the parties agreed upon certain rights and restrictions as set forth therein with respect to the shares purchased by the Investor in accordance with the Prior Purchase Agreement (such shares, the "**Prior Purchased Shares**") and other securities of the Company beneficially owned by the Investor and its Affiliates;

WHEREAS, the Stock Purchase Agreement, of even date herewith, by and between the Investor and the Company (the "**Purchase Agreement**") provides for the issuance and sale by the Company to the Investor, and the purchase by the Investor, of a number of shares (such shares, the "**Newly Purchased Shares**" and, collectively with the Prior Purchased Shares, the "**Purchased Shares**") of the Company's common stock, par value $0.001 per share (the "**Common Stock**");

WHEREAS, as a condition to consummating the transactions contemplated by the Purchase Agreement, the Investor and the Company have agreed upon certain rights and restrictions as set forth herein with respect to the Purchased Shares and other securities of the Company beneficially owned by the Investor and its Affiliates, seek to amend and restate the Prior Investor Agreement in its entirety as set forth herein, and acknowledge that it is a condition to the closing under the Purchase Agreement (the "**Closing**") that this Agreement be in full force and effect; and

WHEREAS, simultaneously with the execution of the Purchase Agreement and this Agreement, the Company and the Investor entered into the Collaboration Agreement.

NOW, THEREFORE, in consideration of the premises and mutual agreements hereinafter set forth, and for other valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree that the Prior Investor Agreement is hereby amended and restated by this Agreement, and that the parties hereto further agree as follows:

1. Definitions. As used in this Agreement, the following terms shall have the following meanings:

(a) "**Affiliate**" shall mean, with respect to any Person, another Person that, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Person. A Person shall be deemed to

control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if such Person (ii) owns, directly or indirectly, beneficially or legally, more than fifty percent (50%) of the outstanding voting securities or capital stock of such other Person, or has other comparable ownership interest with respect to any Person other than a corporation; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

(b) "**Agreement**" shall have the meaning set forth in the Preamble to this Agreement, including all Exhibits attached hereto.

(c) "**Beneficial owner**," "**beneficially owns**," "**beneficial ownership**" and terms of similar import used in this Agreement shall, with respect to a Person, have the meaning set forth in Rule 13d-3 under the Exchange Act (i) assuming the full conversion into, and exercise and exchange for, shares of Common Stock of all Common Stock Equivalents beneficially owned by such Person and (ii) determined without regard for the number of days in which such Person has the right to acquire such beneficial ownership.

(d) "**Board Designation Right Term**" shall mean the period from and after the Closing Date until the occurrence of any event set forth in Section 6.4 hereof.

(e) "**Business Day**" shall mean a day on which banking institutions in Boston, Massachusetts, United States and San Diego, California, United States are open for business, excluding any Saturday or Sunday.

(f) "**Change of Control**" shall mean (i) the acquisition of beneficial ownership, directly or indirectly, by any Third Party of securities or other voting interests of the Company representing a majority of the combined voting power of the Company's then outstanding securities or other voting interests; (ii) any merger, consolidation or business combination involving the Company with a Third Party that results in the holders of beneficial ownership (other than by virtue of obtaining irrevocable proxies) of voting securities or other voting interests of the Company immediately prior to such merger, consolidation or other business combination ceasing to hold beneficial ownership of more than fifty percent (50%) of the combined voting power of the surviving entity immediately after such merger, consolidation or business combination; (iii) any sale, lease, exchange, contribution or other transfer to a Third Party (in one transaction or a series of related transactions) of all or substantially all of the Company's assets; or (iv) individuals who, as of the date hereof, constitute the Board of Directors of the Company (the "**Incumbent Board**") cease for any reason to constitute at least a majority of the Board of Directors of the Company (provided, however, that any individual becoming a director

subsequent to the date hereof whose election, or nomination for election by the Company's shareholders, was recommended or approved by a vote of at least a majority of the directors then comprising the Incumbent Board shall be considered as though such individual were a member of the Incumbent Board, but excluding, for this purpose, any such individual whose initial assumption of office occurs as a result of an actual or threatened election contest with respect to the election or removal of directors or other actual or threatened solicitation of proxies or consents by or on behalf of any person other than the Board of Directors of the Company).

(g) "**Closing Date**" shall have the meaning set forth in the Purchase Agreement.

(h) "**Collaboration Agreement**" shall mean the Collaboration and License Agreement, of even date herewith, between the Investor and the Company.

(i) "**Collaboration Agreement Competitor**" shall mean any operating company with a biopharmaceutical business involving the Development and/or Commercialization of any Competitive Product (as such terms are defined in the Collaboration Agreement), or any other Person that directly or indirectly beneficially owns a majority of the voting securities of or voting interests in such a company, or any direct or indirect majority-owned subsidiary of such a company or of such a Person.

(j) "**Common Stock**" shall have the meaning set forth in the Preamble to this Agreement.

(k) "**Common Stock Equivalents**" shall mean any options, restricted stock units, warrants or other securities or rights convertible into or exercisable, exchangeable or settleable for, whether directly or following conversion into or exercise, exchange or settlement for other options, restricted stock units, warrants or other securities or rights, shares of Common Stock or any swap, hedge or similar agreement or arrangement that transfers in whole or in part, the economic risk of ownership of, or voting or other rights of, the Common Stock.

(l) "**Company**" shall have the meaning set forth in the Preamble to this Agreement.

(m) "**Competitor**" shall mean any Prior Collaboration Agreement Competitor or Collaboration Agreement Competitor.

(n) "**Disposition**," "**Dispose of**" or "**Disposing**" shall mean any (i) pledge, sale, contract to sell, sale of any option or contract to purchase, purchase of any option or contract to sell, grant of any option, right or warrant for the sale of, or other disposition of or transfer of any shares of Common Stock, or any Common Stock Equivalents, including, without limitation, any "short sale" or similar arrangement, or (ii) swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of shares of Common Stock, whether any such swap or transaction is to be settled by delivery of securities, in cash or otherwise.

(o) "**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(p) "**Extraordinary Matter**" shall have the meaning set forth in Section 4.2 hereof.

(q) "**Governmental Authority**" shall mean any multinational, federal, national, state, provincial, local or other entity, office, commission, bureau, agency, political subdivision, instrumentality, branch, department, authority, board, court, arbitral or other tribunal exercising executive, judicial, legislative, police, regulatory, administrative or taxing authority or functions of any nature pertaining to government.

(r) "**Investor**" shall have the meaning set forth in the Preamble to this Agreement.

(s) "**Irrevocable Proxy**" shall have the meaning set forth in Section 4.1 hereof.

(t) "**Law**" shall mean any law, statute, rule, regulation, order, judgment or ordinance having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

(u) "**Lock-Up Agreement**" shall have the meaning set forth in Section 3.4 hereof.

(v) "**Lock-Up Term**" shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 6.2 hereof.

(w) "**Modified Clause**" shall have the meaning set forth in Section 7.6 hereof.

(x) "**Permitted Transferee**" shall mean (i) a controlled Affiliate of the Investor that is wholly owned, directly or indirectly, by the Investor, or (ii) a controlling Affiliate of the Investor (or any controlled Affiliate of such controlling Affiliate) that wholly owns, directly or indirectly, the Investor, or the acquiring Person in the case of a Change of Control of the Investor (replacing references to "Company" with "Investor" in the definition of "Change of Control"); it being understood that for purposes of this definition "wholly owned" shall mean an Affiliate in which the Investor owns, or an Affiliate that owns, as applicable, directly or indirectly, at least ninety-nine percent (99%) of the outstanding capital stock of such Affiliate or the Investor, as applicable.

(y) "**Permitted Transferee Irrevocable Proxy**" shall have the meaning set forth in Section 4.1 hereof.

(z) "**Person**" shall mean any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, Governmental Authority or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

(aa) "**Prior Collaboration Agreement**" shall have the meaning set forth in the Preamble to this Agreement.

(bb) "**Prior Collaboration Agreement Competitor**" shall mean any operating company with a biopharmaceutical business involving the Development and/or Commercialization of any Competitive Product (as such terms are defined in the Prior Collaboration Agreement), or any other Person that directly or indirectly beneficially owns a majority of the voting securities of or voting interests in such a company, or any direct or indirect majority-owned subsidiary of such a company or of such a Person.

(cc) "**Prior Investor Agreement**" shall have the meaning set forth in the Preamble to this Agreement.

(dd) "**Prior Purchase Agreement**" shall have the meaning set forth in the Preamble to this Agreement.

(ee) "**Prior Purchased Shares**" shall have the meaning set forth in the Preamble to this Agreement, and shall be adjusted for (i) any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Prior Purchased Shares.

(ff) "**Purchase Agreement**" shall have the meaning set forth in the Preamble to this Agreement, and shall include all Exhibits attached thereto.

(gg) "**Purchased Shares**" shall have the meaning set forth in the Preamble to this Agreement, and shall be adjusted for (i) any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Purchased Shares.

(hh) "**SEC**" shall mean the U.S. Securities and Exchange Commission.

(ii) "**Securities Act**" shall mean the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

(jj) "**Shares of Then-Outstanding Common Stock**" shall mean, at any time, the issued and outstanding shares of Common Stock at such time, as well as all capital stock issued and outstanding as a result of any stock split, stock dividend, or reclassification of Common Stock distributable, on a pro rata basis, to all holders of Common Stock.

(kk) "**Standstill and Lock-Up Relaxation Date**" shall mean the second anniversary of the Closing Date.

(ll) "**Standstill Parties**" shall have the meaning set forth in Section 2.1 hereof.

(mm) "**Standstill Period**" shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 6.1 hereof.

(nn) "**Third Party**" shall mean any Person other than the Investor, the Company or any Affiliate of the Investor or the Company.

(oo) "**Voting Agreement Term**" shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 6.3 hereof.

2. Restrictions on Beneficial Ownership.

2.1 For the duration of the Standstill Period, unless the Company or its Affiliates or representatives have specifically invited or approved the Investor to do so in writing, neither the Investor nor any of its Affiliates or representatives acting on behalf of the Investor (collectively, the "**Standstill Parties**") will in any manner, directly or indirectly: (i) effect or seek, offer or propose (whether publicly or otherwise) to effect, or cause or knowingly participate in or in any way advise, assist or knowingly encourage any other Person to effect or seek, offer or propose (whether publicly or otherwise) to effect or participate in, (A) any acquisition of any securities (or beneficial ownership thereof) or assets of the Company, or any rights to acquire any such securities (including derivative securities representing the right to vote or economic benefit of any such securities) or assets; (B) any tender or exchange offer, merger or other business combination involving the Company; (C) any recapitalization, restructuring, liquidation, dissolution or other extraordinary transaction with respect to the Company; or (D) any "solicitation" of "proxies" (as such terms are used in the proxy rules of the SEC) or consents to vote any voting securities of the Company; (ii) form, join or in any way participate in a "group" (as defined under the Exchange Act) with respect to any securities of the Company; (iii) otherwise act, alone or in concert with others, to seek to control or influence the management, Board of Directors or policies of the Company; (iv) take any action that would reasonably be expected to require the Company to make a public announcement regarding any of the types of matters set forth in clause (i) above; (v) enter into any discussions or arrangements with any Third Party other than Investor's advisors with respect to any of the foregoing; or (vi) publicly disclose any intention, plan or arrangement regarding any of the foregoing. Notwithstanding anything to the contrary contained in this Agreement, Investor and its Affiliates shall not be precluded from owning or acquiring interests in mutual funds or similar entities that own capital stock of the Company, and nothing herein shall prohibit passive investments by pension or employee benefit plans of Investor.

2.2 The Investor also agrees during the Standstill Period not to request the Company (or its directors, officers, employees or agents), directly or indirectly, to amend or waive any provision of this Section 2 (including this sentence).

2.3 Notwithstanding anything to the contrary contained in this Agreement, if, at any time (i) a Third Party enters into a definitive agreement with the Company contemplating

the acquisition (by way of merger, tender offer or otherwise) of more than fifty percent (50%) of the then-outstanding Common Stock of the Company, of securities representing more than fifty percent (50%) of the voting power of all then-outstanding securities of the Company or all or substantially all of the consolidated assets of the Company or publicly announces its intention to do so, then the restrictions set forth in Section 2.1 shall terminate and cease to be of any further force or effect or (ii) a Third Party commences, or publicly announces an intention to commence, a tender or exchange offer that, if consummated, would make such third party the beneficial owner (within the meaning of Section 13(d)(1) of the Exchange Act) of at least 50% of the voting power of all then-outstanding securities of the Company, then until the expiration or termination of a tender or exchange offer that has been commenced or until the public announcement of a withdrawal or abandonment of an intention to commence a tender or exchange offer, the restrictions set forth in Section 2.1 shall be suspended and of no force or effect.

2.4 Notwithstanding anything to the contrary contained in this Agreement, on and after the Standstill and Lock-Up Relaxation Date, Investor shall not be precluded from making any confidential offers or proposals to the Board of Directors of the Company in a manner reasonably believed not to require the Company to make a public announcement of such offer or proposal; provided, however, that the Investor not disclose its interest or intention to make, or the actual making of, any such offer or proposal.

3. Restrictions on Dispositions.

3.1 Lock-Up. During the Lock-Up Term, without the prior approval of the Company, the Investor shall not, and shall cause its Affiliates not to, Dispose of any of the Purchased Shares; provided, however, that the foregoing shall not prohibit the Investor from (i) transferring the Purchased Shares to a Permitted Transferee in accordance with the terms hereof or (ii) Disposing of any Purchased Shares to reduce the beneficial ownership of the Standstill Parties to nineteen and ninety-nine hundredths percent (19.99%) of the Shares of Then-Outstanding Common Stock; and provided further that, notwithstanding anything in this Section 3.1, the Investor shall not be precluded from the Disposition of Purchased Shares through open market sales effected through one or more "brokers' transactions" (as such term is used in Rule 144 promulgated under the Securities Act) on or after the Standstill and Lock-Up Relaxation Date in an amount not to exceed one percent (1%) of the Shares of Then-Outstanding Common Stock in any three (3) month period.

3.2 Certain Tender Offers. Subject to the restrictions set forth in Section 3.3 hereof, this Section 3 shall not prohibit or restrict any Disposition of Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents by the Standstill Parties into (i) a tender offer by a Third Party or (ii) an issuer tender offer by the Company.

3.3 Sale Limitations. Subject to the restrictions set forth in Section 3.1 hereof, the Investor agrees that, except for any transfer of Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents by the Investor to a Permitted Transferee in accordance with the terms hereof or the Company, it (i) shall not, and shall cause its Affiliates not to, Dispose of any Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents, in a "block trade" private placement transaction, at any time to any Person that such Investor or Affiliate knows (after a reasonable inquiry) is a Competitor of the Company and (ii) shall, and shall cause

its Affiliates to, instruct the broker(s) in any such "block trade" not to Dispose Shares to a Competitor (unless the identity of the Person purchasing the Shares is not known to the broker(s) or such Person Disposing of Shares).

3.4 Offering Lock-Up. The Investor shall, if requested by the Company and an underwriter of Common Stock of the Company in connection with any public offering involving an underwriting of Common Stock of the Company (whether such public offering takes place before or after the expiration of the Lock-Up Term), agree not to Dispose of any Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents for a specified period of time immediately following the launch of such offering (the "**Lock-Up Period**"), such period of time not to exceed ninety (90) days following the pricing of such offering (a "**Lock-Up Agreement**"), provided that all officers and directors of the Company are subject to the same restrictions, and provided, further, that such Lock-Up Agreement shall not restrict the Investor's ability to Dispose of any Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents in accordance with Section 3.2 hereof during the Lock-Up Term. Any Lock-Up Agreement shall be in writing in a form reasonably satisfactory to the Company and the underwriter(s) in such offering. The Company may impose stop transfer instructions with respect to the Shares of Then-Outstanding Common Stock and/or Common Stock Equivalents subject to the foregoing restrictions until the end of the Lock-Up Term. Any discretionary waiver or termination of the restrictions of any or all of such Lock-Up Agreements by the Company or the underwriters shall apply pro rata to the Investor based on the number of shares subject to such Lock-Up Agreements, excluding any waivers granted that fall within a customary de minimis exemption set forth in the associated Lock-Up Agreement.

3.5 Transactions for Personal Account; Change of Control of the Investor. For the avoidance of doubt, nothing in this Section 3 will restrict any Disposition of shares of Common Stock (i) held by an executive officer or director of the Investor for his or her personal account or (ii) that may occur (or be deemed to occur) in connection with a Change of Control of the Investor (replacing references to "Company" with "Investor" in the definition of "Change of Control").

4. Voting Agreement.

4.1 Voting of Securities. During the Voting Agreement Term, other than as permitted by Section 4.2 hereof with respect to Extraordinary Matters, in any vote or any action by written consent of the stockholders of the Company (including, without limitation, with respect to the election of directors), the Investor shall, and shall cause any Permitted Transferees to, vote or execute a written consent with respect to the Purchased Shares, in the sole discretion of the Investor, in accordance with the recommendation of the Company's Board of Directors. In furtherance of this Section 4.1, the Investor hereby irrevocably appoints the Company and any individuals designated by the Company (such designated individuals to be limited to the President and Chief Executive Officer, the Chief Financial Officer, the Chief Operating Officer, the Senior Vice President and General Counsel, and the Secretary of the Company), and each of them individually, as the attorneys, agents and proxies, with full power of substitution and resubstitution in each of them, for the Investor, and in the name, place and stead of the Investor, to vote (or cause to be voted) in such manner as set forth in this Section 4.1 (but in any case, excluding any matter that is an Extraordinary Matter described in Section 4.2 hereof) with respect to the Purchased Shares to which the Investor is or may be entitled to vote at any meeting of the Company held after

the date hereof, whether annual or special and whether or not an adjourned meeting (the "**Irrevocable Proxy**"). This Irrevocable Proxy is coupled with an interest, shall be irrevocable and binding on any successor-in-interest of the Investor and shall not be terminated by operation of Law upon the occurrence of any event. This Irrevocable Proxy shall operate to revoke and render void any prior proxy as to voting securities heretofore granted by the Investor which is inconsistent herewith. Notwithstanding the foregoing, the Irrevocable Proxy shall be effective only if, at any annual or special meeting of the stockholders of the Company and at any adjournments or postponements of any such meetings, the Investor (i) fails to appear or otherwise fails to cause its voting securities of the Company to be counted as present for purposes of calculating a quorum, or (ii) fails to vote such voting securities in accordance with this Section 4.1, in each case at least five (5) Business Days prior to the date of such stockholders' meeting. The Irrevocable Proxy shall terminate upon the earlier of the expiration or termination of the Voting Agreement Term. The Investor shall cause any Permitted Transferee to promptly execute and deliver to the Company an irrevocable proxy, substantially in the form of Exhibit A attached hereto, and irrevocably appoint the Company and any individuals designated by the Company, and each of them individually, with full power of substitution and resubstitution, as the attorneys, agents and proxies to vote (or cause to be voted) such Purchased Shares of the Company as to which such Permitted Transferee is entitled to vote, in such manner as each such attorney, agent and proxy or its, his or her substitute shall in its, his or her sole discretion deem appropriate or desirable with respect to the matters set forth in this Section 4.1 (the "**Permitted Transferee Irrevocable Proxy**"). The Investor acknowledges, and shall cause any Permitted Transferees to acknowledge, that any such proxy executed and delivered shall be coupled with an interest, shall constitute, among other things, an inducement for the Company to enter into this Agreement, shall be irrevocable and binding on any successor-in-interest of such Permitted Transferee and shall not be terminated by operation of Law upon the occurrence of any event. Such proxy shall operate to revoke and render void any prior proxy as to any voting securities of the Company heretofore granted by such Permitted Transferee, to the extent it is inconsistent herewith. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to such Permitted Transferee that such Permitted Transferee execute and deliver to the Company a Permitted Transferee Irrevocable Proxy, and that any purported transfer shall be void and of no force or effect if such Permitted Transferee Irrevocable Proxy is not so executed and delivered at the closing of such transfer. Such proxy shall terminate upon the earlier of the expiration or termination of the Voting Agreement Term. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to any Permitted Transferee during the Voting Agreement Term that such Permitted Transferee shall agree in writing to be subject to and bound by all restrictions and obligations set forth in this Section 4.1.

In the event the Company's stockholders are permitted to act by written consent, the Company and the Investor shall each negotiate in good faith with the other provisions as consistent as possible with the foregoing to govern the voting of the Investor's and its Permitted Transferees' Shares of Then-Outstanding Common Stock as closely as practicable to the foregoing.

4.2 Certain Extraordinary Matters. The Investor and its Permitted Transferees may vote, or execute a written consent with respect to, any or all of the voting securities of the Company as to which they are entitled to vote or execute a written consent, as they may determine in their sole discretion, with respect to the following matters (each such matter being an

"**Extraordinary Matter**"):

(a) any transaction which would result in a Change of Control of the Company; and

(b) any liquidation or dissolution of the Company.

4.3 Quorum. In furtherance of Section 4.1 hereof, the Investor shall be, and shall cause each of its Permitted Transferees to be, present in person or represented by proxy at all meetings of stockholders to the extent necessary so that all voting securities of the Company as to which they are entitled to vote shall be counted as present for the purpose of determining the presence of a quorum at such meeting.

5. Board Matters.

5.1 Board Seat. The Company shall cause Jude Onyia, Ph.D., to be appointed to the Company's Board of Directors as a Class III director, contingent upon and effective as of the Closing Date, with an initial term expiring at the 2024 Annual Meeting of Stockholders of the Company (the "**Initial NBIX Director**"). For the duration of the Board Designation Right Term, the Company shall cause Dr. Onyia or another individual designated by the Investor and reasonably acceptable to the Company's Board of Directors (as applicable, a **"Subsequent NBIX Director"** and, with the Initial NBIX Director, each an "**NBIX Director**") to be nominated for election to the Company's Board of Directors at subsequent Annual Meeting(s) of Stockholders of the Company for a successive term commencing upon the expiration of the then-serving NBIX Director's term, provided that the Company's Board of Directors determines in good faith and consistent with such directors' fiduciary duties that such nominee meets the minimum qualifications established for director nominees as set forth in the Company's Corporate Governance Guidelines as then in effect. Each NBIX Director shall (i) be subject to all of the Company's policies, procedures, processes, codes, standards, guidelines and rules generally applicable to the Company's directors and (ii) as a condition to his or her nomination and election to the Board of Directors, complete the Company's standard director and officer questionnaire and furnish other reasonable and customary director documentation and information reasonably requested by the Company in connection with the election of members of the Company's Board of Directors and generally applicable to the Company's directors.

5.2 Removal; Resignation. Promptly following, but in no event more than five Business Days after, the expiration or termination of the Board Designation Right Term, the Investor shall cause the removal or resignation of the NBIX Director, effective immediately. In connection with the appointment or election of any NBIX Director, the Investor shall enter into a written agreement with such NBIX Director whereby the NBIX Director agrees to resign as a member of the Board of Directors in connection with the expiration or termination of the Board Designation Right Term.

6. Termination of Certain Rights and Obligations.

6.1 Termination of Standstill Period. Section 2 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the later of (x) the expiration or earlier valid termination of the Prior Collaboration Agreement and (y) the expiration or earlier valid termination of the Collaboration Agreement;

(b) the date that is the third anniversary of the Closing Date;

(c) a liquidation or dissolution of the Company; and

(d) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act.

6.2 Termination of Lock-Up Term. Section 3.1 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the date that is the third anniversary of the Closing Date;

(b) the beneficial ownership of the Standstill Parties falls below three percent (3%) of the Shares of Then-Outstanding Common Stock;

(c) a Change of Control of the Company;

(d) a liquidation or dissolution of the Company; and

(e) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act.

6.3 Termination of Voting Agreement Term. Section 4 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the date that is the third anniversary of the Closing Date;

(b) the beneficial ownership of the Standstill Parties falls below three percent (3%) of the Shares of Then-Outstanding Common Stock;

(c) a Change of Control of the Company;

(d) the later of (x) the expiration or earlier valid termination of the Prior Collaboration Agreement and (y) the expiration or earlier valid termination of the Collaboration Agreement; and

(e) a liquidation or dissolution of the Company.

6.4 Termination of Board Designation Right Term. Section 5.1 hereof shall terminate and have no further force or effect upon the earliest to occur of:

(a) the date that is the tenth anniversary of the Closing Date;

(b) a Change of Control of the Company;

(c) a Change of Control of the Investor (replacing references to "Company" with "Investor" in the definition of "Change of Control");

(d) the beneficial ownership of the Investor falling below ten percent (10.0%) of the Shares of Then-Outstanding Common Stock; and

(e) a liquidation or dissolution of the Company.

6.5 Termination of Agreement. This Agreement shall terminate and have no further force or effect upon any termination of the Purchase Agreement prior to the Closing pursuant to Section 10.1 thereof. In the event of such a termination, the Prior Investor Agreement shall be automatically deemed reinstated, with retroactive effect to the date of this Agreement, as if it had never been amended, restated, superseded or replaced hereby.

6.6 Effect of Termination. No termination pursuant to any of Sections 6.1, 6.2, 6.3, 6.4, or 6.5 hereof shall relieve any of the parties (or the Permitted Transferee, if any) for liability for breach of or default under any of their respective obligations or restrictions under any terminated provision of this Agreement, which breach or default arose out of events or circumstances occurring or existing prior to the date of such termination.

7. Miscellaneous.

7.1 Governing Law; Submission to Jurisdiction. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Any action brought, arising out of, or relating to this Agreement shall be brought in the Court of Chancery of the State of Delaware. Each party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The parties hereby consent to and grant the Court of Chancery of the State of Delaware jurisdiction over such parties and over the subject matter of any such claim and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 7.3 hereof or in such other manner as may be permitted by law, shall be valid and sufficient thereof.

7.2 Waiver. Neither party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either party to assert a right hereunder or to insist upon compliance with any term of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term except to the extent set forth in writing.

7.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit B attached hereto and shall be (i) delivered personally; (ii) sent by certified mail (return receipt requested), postage prepaid; or (iii) sent via a reputable nationwide overnight express courier service (signature required). Any such notice, instruction or communication shall be deemed to have been delivered (A) upon receipt if delivered by hand; (B) three (3) Business Days after it is sent by certified mail, return receipt requested, postage prepaid; or (C) one (1) Business Day after it is sent via a reputable nationwide overnight courier service. Either party may change its address by giving notice to the other party in the manner provided above; provided that notices of a change of address shall be effective only upon receipt thereof.

7.4 Entire Agreement. This Agreement, the Purchase Agreement and the Collaboration Agreement, in each case together with the schedules and exhibits thereto, set forth all the covenants, promises, agreements, warranties, representations, conditions and understandings between the parties and supersede and terminate all prior agreements and understanding between the parties. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the parties other than as set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the parties unless reduced to writing and signed by the respective authorized officers of the parties. Upon the effectiveness of this Agreement, but subject to Section 6.5 hereof, the Prior Investor Agreement shall be deemed amended, restated, superseded and replaced in its entirety by this Agreement and shall be of no further force or effect, and any reference to the Prior Investor Agreement in the Prior Collaboration Agreement or the Prior Purchase Agreement shall mean and be a reference to this Agreement, as may be amended and/or restated from time to time.

7.5 Headings; Nouns and Pronouns; Section References. Headings and any table of contents used in this Agreement are for convenience only and shall not in any way affect the construction of or be taken into consideration in interpreting this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

7.6 Severability. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

7.7 Assignment. Except for an assignment of this Agreement by the Investor to a Permitted Transferee, neither this Agreement nor any rights or duties of a party hereto may be assigned by such party, in whole or in part, without (i) the prior written consent of the Company

in the case of any assignment by the Investor; or (ii) the prior written consent of the Investor in the case of an assignment by the Company.

7.8 Parties in Interest. All of the terms and provisions of this Agreement shall be binding upon, and shall inure to the benefit of and be enforceable by the parties hereto and their respective successors, heirs, administrators and permitted assigns.

7.9 Counterparts. This Agreement may be signed in counterparts, each and every one of which shall be deemed an original, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

7.10 Third-Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto. No Third Party with the exception of any Affiliate of the Investor shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

7.11 No Strict Construction. This Agreement has been prepared jointly and will not be construed against either party.

7.12 Remedies. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

7.13 Specific Performance. The Company and the Investor hereby acknowledge and agree that the rights of the parties hereunder are special, unique and of extraordinary character, and that if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or failure would result in irreparable injury to the Company or the Investor, as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, then, in addition to any other remedy which may be available to any damaged party at law or in equity, such damaged party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged party will be entitled to seek in any court of competent jurisdiction.

7.14 No Conflicting Agreements. The Investor hereby represents and warrants to the Company that neither it nor any of its Affiliates is, as of the date of this Agreement, a party to, and agrees that neither it nor any of its Affiliates shall, on or after the date of this Agreement, enter into any agreement that conflicts with the rights granted to the Company in this Agreement. The Company hereby represents and warrants to the Investor that it is not, as of the date of this Agreement, a party to, and agrees that it shall not, on or after the date of this Agreement, enter into

any agreement or approve any amendment to its charter or by-laws or similar organizational documents of the Company with respect to its securities that conflicts with the rights granted to the Investor in this Agreement which have not expired or been terminated in accordance with the terms hereof. The Company further represents and warrants that the rights granted to the Investor hereunder do not in any way conflict with the rights granted to any other holder of the Company's securities under any other agreements.

7.15 Use of Proceeds. The Company shall use the proceeds from the sale of the Purchased Shares for research and development and other working capital purposes and shall not use such proceeds for the redemption of any shares of Common Stock or for the payment of any dividends on shares of Common Stock.

7.16 No Publicity. The parties hereto agree that the provisions of Section 11.3 of the Collaboration Agreement shall be applicable to the parties to this Agreement with respect to any public disclosures regarding the proposed transactions contemplated by the Purchase Agreement and the Collaboration Agreement or regarding the parties hereto or their Affiliates (it being understood that the provisions of Section 11.3 of the Collaboration Agreement shall be read to apply to disclosures of information relating to this Agreement and the transactions contemplated hereby).

(Signature Page Follows)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

NEUROCRINE BIOSCIENCES, INC.

By: /s/ Kevin Gorman
Name: Kevin Gorman
Title: CEO

VOYAGER THERAPEUTICS, INC.

By: /s/ Alfred W. Sandrock, Jr.
Name: Alfred W. Sandrock, Jr., M.D., Ph.D.
Title: President & CEO

[*Signature Page to Investor Agreement*]

EXHIBIT A

FORM OF IRREVOCABLE PROXY

To secure the performance of the duties of the undersigned pursuant to Section 4.1 of the Investor Agreement, dated as of January 8, 2023 (the "**Agreement**"), by and between Neurocrine Biosciences, Inc. and Voyager Therapeutics, Inc. (the "**Company**"), the undersigned hereby irrevocably appoints the Company and any individual designated by the Company, and each of them individually, as the attorneys, agents and proxies, with full power of substitution and resubstitution in each of them, for the undersigned, and in the name, place and stead of the undersigned, to vote (or cause to be voted) in such manner as set forth in Section 4.1 of the Agreement (but in any case excluding any matter that is an Extraordinary Matter described in Section 4.2) with respect to all Purchased Shares, which the undersigned is or may be entitled to vote at any meeting of the Company held after the date hereof, whether annual or special and whether or not an adjourned meeting. This proxy is coupled with an interest, shall be irrevocable and binding on any successor-in-interest of the undersigned and shall not be terminated by operation of Law upon the occurrence of any event. This proxy shall operate to revoke and render void any prior proxy as to voting securities heretofore granted by the undersigned which is inconsistent herewith. Notwithstanding the foregoing, this irrevocable proxy shall be effective only if, at any annual or special meeting of the stockholders of the Company (or any consent in lieu thereof) and at any adjournments or postponements of any such meetings, the undersigned (A) fails to appear or otherwise fails to cause its voting securities of the Company to be counted as present for purposes of calculating a quorum, or (B) fails to vote such voting securities in accordance with Section 4.1 of the Agreement, in each case at least five (5) Business Days prior to the date of such stockholders' meeting. This proxy shall terminate upon the earlier of the expiration or termination of the Voting Agreement Term. Capitalized terms used but not defined herein shall have the meanings given them in the Agreement.

NEUROCRINE BIOSCIENCES, INC.

By: _____
Name:
Title:

EXHIBIT B

NOTICES

If to the Investor:

Neurocrine Biosciences, Inc.
12780 El Camino Real
San Diego, CA 92130
Attention: General Counsel

with a copy to:

Cooley LLP
55 Hudson Yards
New York, NY 10001
Attention: Jason L. Kent, Esq.

If to the Company:

Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: Chief Executive Officer

with copies to:

Voyager Therapeutics, Inc.
64 Sidney Street
Cambridge, MA 02139
Attention: General Counsel

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, NY 10007
Attention: Brian A. Johnson, Esq.

Exhibit 21.1

SUBSIDIARIES OF THE REGISTRANT

Name of Entity	State/Country of Organization
Voyager Securities	Corporation Massachusetts

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

- Registration Statement (Form S-3 No. 333-268240) and related Prospectus of Voyager Therapeutics, Inc. for the registration of common stock, preferred stock, debt securities, depositary shares, subscription rights, warrants, purchase contracts, and units,
- Registration Statement (Form S-8 No. 333-207958) pertaining to the 2014 Stock Option and Grant Plan, the 2015 Stock Option and Incentive Plan, and the 2015 Employee Stock Purchase Plan of Voyager Therapeutics, Inc.,
- Registration Statement (Form S-8 Nos. 333-210258, 333-216699, 333-223638, 333-236870 and 333-263356) pertaining to the 2015 Stock Option and Incentive Plan and the 2015 Employee Stock Purchase Plan of Voyager Therapeutics, Inc., and
- Registration Statement (Form S-8 Nos. 333-229891 and 333-253549) pertaining to the 2015 Stock Option and Incentive Plan, the 2015 Employee Stock Purchase Plan, the Inducement Stock Option Grant Awards and the Inducement Restricted Stock Unit Awards of Voyager Therapeutics, Inc.;

of our report dated March 7, 2023, with respect to the consolidated financial statements of Voyager Therapeutics, Inc. included in this Annual Report (Form 10-K) of Voyager Therapeutics, Inc. for the year ended December 31, 2022.

/s/ Ernst & Young LLP

Boston, Massachusetts
March 7, 2023

Exhibit 31.1

Certification

I, Alfred Sandrock, certify that:

1. I have reviewed this Annual Report on Form 10-K of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 7, 2023

/s/Alfred Sandrock, M.D., Ph.D.
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Exhibit 31.2

Certification

I, Peter P. Pfreundschuh, certify that:

1. I have reviewed this Annual Report on Form 10-K of Voyager Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 7, 2023

/s/Peter P. Pfreundschuh
Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Voyager Therapeutics, Inc. (the "Company") for the year ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his or her knowledge:

1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 07, 2023

/s/ Alfred Sandrock, M.D., Ph.D.
Alfred Sandrock, M.D., Ph.D.
Chief Executive Officer, President, and Director
(Principal Executive Officer)

Date: March 07, 2023

/s/Peter P. Pfreundschuh
Peter P. Pfreundschuh
Chief Financial Officer
(Principal Financial and Accounting Officer)