



## Voyager Therapeutics Reports First Quarter 2024 Financial and Operating Results

05/13/24

- Company announces clearance of IND application with FDA for anti-tau antibody VY-TAU01 for the treatment of Alzheimer's disease; expect to begin single ascending dose trial in the coming weeks -
- Development candidates selected for Neurocrine-partnered GBA1 and Friedreich's Ataxia gene therapy programs; potential for three gene therapies, including SOD1-ALS, to enter the clinic in 2025 -
  - Appointed neurology clinical development expert Toby Ferguson, M.D., Ph.D., as Chief Medical Officer -
- Strong cash position of approximately \$400 million as of March 31, 2024; expected to provide runway through multiple clinical data readouts into 2027 -

LEXINGTON, Mass., May 13, 2024 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to advancing neurogenetic medicines, today reported first quarter 2024 financial and operating results.

"We have obtained IND clearance for our anti-tau antibody VY-TAU01 for Alzheimer's disease, and we expect to dose the first subject in our single ascending dose trial in healthy volunteers in the coming weeks," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "Our gene therapy pipeline is also advancing, with development candidates selected in the GBA1 and Friedreich's Ataxia programs partnered with Neurocrine, as well as in our wholly-owned SOD1-ALS program. We expect to achieve IND filings for all three of these gene therapy programs in 2025. We maintain a strong cash position of approximately \$400 million at quarter-end, with runway into 2027, which we anticipate will enable us to reach multiple data readouts in 2025 and 2026."

### First Quarter 2024 and Recent Highlights

- **Obtained IND clearance for VY-TAU01 for Alzheimer's disease:** Received clearance of the Investigational New Drug (IND) application filed with the U.S. Food and Drug Administration (FDA) for VY-TAU01, an anti-tau antibody for the treatment of Alzheimer's disease.
- **Development candidate selected in GBA1 gene therapy program:** Announced that the joint steering committee with collaborator Neurocrine Biosciences selected a lead development candidate for the GBA1 gene therapy program for the treatment of Parkinson's disease and other GBA1-mediated diseases, triggering a \$3 million milestone payment to Voyager.
- **Development candidate selected in FXN gene therapy program:** Announced that the joint steering committee with collaborator Neurocrine Biosciences selected a lead development candidate for the FXN gene therapy program for the treatment of Friedreich's Ataxia, triggering a \$5 million milestone payment to Voyager.
- **Toby Ferguson, M.D., Ph.D., appointed as Chief Medical Officer:** Dr. Ferguson is an experienced biotechnology executive with a proven record in advancing portfolios of novel therapies across diverse indications in central nervous system (CNS), neuromuscular, and rare diseases.
- **Strategic collaboration and capsid license agreement with Novartis:** Announced a strategic collaboration and capsid license agreement with Novartis Pharma AG to advance potential gene therapies for Huntington's disease (HD) and spinal muscular atrophy (SMA). Novartis paid Voyager \$80 million of consideration up front and \$20 million for the purchase of newly issued equity in Voyager. Voyager is eligible to receive up to \$1.2 billion in preclinical, development, regulatory and sales milestones, as well as tiered royalties on global net sales of products incorporating Voyager's TRACER™ capsids.
- **Completion of public offering:** Completed an underwritten public offering of shares of common stock (or pre-funded warrants to purchase common stock in lieu thereof) for aggregate gross proceeds of approximately \$100 million.
- **Presented data at AD/PD 2024 and ASGCT 2024:**
  - *2024 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD™ 2024)* Presented data demonstrating robust reductions in human tau messenger RNA (mRNA) and protein across the brain following a single intravenous (IV) administration of a tau silencing gene therapy candidate in mice expressing human tau. Also presented data demonstrating that VY-TAU01 was well-tolerated following IV administration in non-human primates (NHPs).
  - *American Society of Gene & Cell Therapy's (ASGCT) 27th annual meeting:* Presented a broad set of translational data, including new data demonstrating enhanced brain tropism of second-generation TRACER-derived AAV capsids, translatability as evidenced by cross-species and receptor data; and activity against therapeutic targets in Alzheimer's disease and ALS.

## Anticipated Upcoming Milestones

- **VY-TAU01 anti-tau antibody for Alzheimer's disease:** Voyager expects to dose the first subject in a Phase 1a single ascending dose trial in healthy volunteers in the coming weeks and initiate a Phase 1b multiple ascending dose trial in patients with early Alzheimer's disease in 2025. The multiple ascending dose trial has the potential to generate initial data for slowing the spread of pathological tau via tau PET imaging in 2026.
- **VY9323 SOD1 silencing gene therapy program for ALS:** Voyager expects to file an IND in mid-2025 and initiate a Phase 1 clinical trial in ALS patients. The Phase 1 trial has the potential to generate proof-of-concept data based on biomarkers.
- **Partnered programs:** Voyager and Neurocrine Biosciences expect to file INDs for the GBA1 and Friedreich's Ataxia gene therapy programs in 2025.

## First Quarter 2024 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$19.5 million for the first quarter of 2024, compared to \$150.5 million for the same period in 2023. The decrease was primarily due to the first quarter 2023 recognition of \$69.5 million from the 2023 Neurocrine Collaboration Agreement and \$79.0 million from Novartis' exercise of two capsid options.
- **Net (Loss) Income:** Net loss was \$11.3 million for the first quarter of 2024, compared to net income of \$124.0 million for the same period in 2023. The decrease is primarily due to reduced collaboration revenue recognized in the first quarter of 2024, as discussed above.
- **R&D Expenses:** Research and development expenses were \$27.1 million for the first quarter of 2024, compared to \$18.6 million for the same period in 2023. The increase in R&D expenses was primarily a result of increased program-related spending, particularly manufacturing and IND-enabling studies for the VY-TAU01 anti-tau antibody program, the VY9323 SOD1-ALS gene therapy program, and the initiation of spend on the Novartis HD program during the first quarter of 2024, along with increased headcount costs.
- **G&A Expenses:** General and administrative expenses were \$8.6 million for the first quarter of 2024, compared to \$9.0 million for the same period in 2023.
- **Cash Position:** Cash, cash equivalents and marketable securities as of March 31, 2024, were \$400.5 million.

## Financial Guidance

Voyager is committed to maintaining a strong balance sheet that supports the advancement and growth of its platform and pipeline. Voyager continues to assess its planned cash needs both during the current period and in future periods. We expect our cash, cash equivalents, and marketable securities, along with amounts expected to be received as reimbursement for development costs under the Neurocrine and Novartis collaborations, certain near-term milestones, and interest income, to be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2027.

## Conference Call

Voyager will host a conference call and webcast today at 4:30 p.m. ET to discuss first quarter 2024 financial and operating results. To participate via telephone and join the call live, please register in advance here: <https://register.vevent.com/register/B11f6af80e7a614ca7925cbad2f35a55c6>. Upon registration, telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number and a unique passcode. A live webcast of the call will also be available on the Investors section of the Voyager website at [ir.voyagertherapeutics.com](http://ir.voyagertherapeutics.com), and a replay of the call will be available at the same link approximately two hours after its completion. The replay will be available for at least 30 days following the conclusion of the call.

## About the TRACER™ Capsid Discovery Platform

Voyager's TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) capsid discovery platform is a broadly applicable, RNA-based screening platform that enables rapid discovery of novel AAV capsids to enable gene therapy. Voyager has leveraged TRACER to create multiple families of novel capsids that, following intravenous delivery in preclinical studies, harness the extensive vasculature of the central nervous system (CNS) to cross the blood-brain barrier and transduce a broad range of CNS regions and cell types. In cross-species preclinical studies (rodents and multiple non-human primate species), intravenous delivery of TRACER-generated capsids resulted in widespread payload expression across the CNS at relatively low doses, enabling selection of multiple development candidates in Voyager's wholly-owned and partnered gene therapy programs for neurologic diseases.

## About Voyager Therapeutics

Voyager Therapeutics, Inc. (Nasdaq: VYGR) is a biotechnology company dedicated to leveraging the power of human genetics to modify the course of – and ultimately cure – neurological diseases. Our pipeline includes programs for Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Parkinson's disease, and multiple other diseases of the central nervous system. Many of our programs are derived from our TRACER™ AAV capsid discovery platform, which we have used to generate novel capsids and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of our programs are wholly owned, and some are advancing with partners including Alexion, AstraZeneca Rare Disease; Novartis Pharma AG; Neurocrine Biosciences, Inc.; and Sangamo Therapeutics, Inc. For more information, visit [www.voyagertherapeutics.com](http://www.voyagertherapeutics.com).

*Voyager Therapeutics® is a registered trademark, and TRACER™ is a trademark, of Voyager Therapeutics, Inc.*

## Forward-Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform

Act of 1995 and other federal securities laws. The use of words such as “expect,” “will,” “believe,” “anticipate,” “potential,” “trigger” or “continue,” and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager’s ability to advance its AAV-based gene therapy programs and tau antibody program, including expectations for Voyager’s achievement of preclinical and clinical development milestones for its potential development candidates such as IND filings, the initiation of clinical trials, and generation of clinical data and proof-of-concept; Voyager’s ability to advance gene therapy product candidates under the Neurocrine and Novartis collaborations; Voyager’s anticipated financial results, including the anticipated receipt by Voyager of revenues or reimbursement payments from collaboration partners; and Voyager’s cash runway and ability to generate sufficient cash resources to enable it to continue its business and operations are forward looking.

All forward-looking statements are based on estimates and assumptions by Voyager’s management that, although Voyager believes such forward-looking statements to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Voyager expected. Such risks and uncertainties include, among others, the expectations and decisions of regulatory authorities; the timing, initiation, conduct and outcomes of Voyager’s preclinical and clinical studies; the availability of data from clinical trials; the availability or commercial potential of product candidates under collaborations; the willingness and ability of Voyager’s collaboration partners to meet obligations under collaboration agreements with Voyager; the continued development of Voyager’s technology platforms, including Voyager’s TRACER platform and its antibody screening technology; Voyager’s scientific approach and program development progress, and the restricted supply of critical research components; the development by third parties of capsid identification platforms that may be competitive to Voyager’s TRACER capsid discovery platform; Voyager’s ability to create and protect intellectual property rights associated with the TRACER capsid discovery platform, the capsids identified by the platform, and development candidates for Voyager’s pipeline programs; the possibility or the timing of Voyager’s receipt of program reimbursement, development or commercialization milestones, option exercise, and other payments under Voyager’s existing licensing or collaboration agreements; the ability of Voyager to negotiate and complete licensing or collaboration agreements with other parties on terms acceptable to Voyager and the third parties; the success of programs controlled by third party collaboration partners in which Voyager retains a financial interest, and the success of Voyager’s product candidates; the ability to attract and retain talented directors, employees, and contractors; and the sufficiency of cash resources to fund its operations and pursue its corporate objectives.

These statements are also subject to a number of material risks and uncertainties that are described in Voyager’s most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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### Selected Financial Information (\$ amounts in thousands, except per share data) (Unaudited)

	Three Months Ended March 31,	
	2024	2023
<b>Statement of Operations Items:</b>		
Collaboration revenue	\$ 19,516	\$ 150,480
Operating expenses:		
Research and development	27,092	18,568
General and administrative	8,607	9,028
Total operating expenses	35,699	27,596
Operating (loss) income	(16,183)	122,884
Total other income	4,867	1,864
(Loss) income before income taxes	(11,316)	124,748
Income tax provision	14	704
Net (loss) income	\$ (11,330)	\$ 124,044
Net (loss) income per share, basic	\$ (0.20)	\$ 3.05
Net (loss) income per share, diluted	\$ (0.20)	\$ 2.94
Weighted-average common shares outstanding, basic	57,117,046	40,632,087
Weighted-average common shares outstanding, diluted	57,117,046	42,161,326

	March 31,	December 31,
	2024	2023
<b>Selected Balance Sheet Items</b>		
Cash, cash equivalents, and marketable securities	\$ 400,548	\$ 230,875
Total assets	\$ 469,592	\$ 351,281
Accounts payable and accrued expenses	\$ 15,174	\$ 18,427
Deferred revenue	\$ 64,596	\$ 75,240
Total stockholders’ equity	\$ 340,886	\$ 236,320

## GAAP vs. Non-GAAP Financial Measures

Voyager’s financial statements are prepared in accordance with generally accepted accounting principles in the United States, or GAAP, and represent revenue and expenses as reported to the Securities and Exchange Commission. Voyager has provided in this release certain financial information that

has not been prepared in accordance with GAAP, including net collaboration revenue and net research and development expenses, which exclude the impact of reimbursement by Neurocrine Biosciences (Neurocrine) for expenses we incur in conducting preclinical development activities under our collaboration agreements. Management uses these non-GAAP measures to evaluate the Company's operating performance in a manner that allows for meaningful period-to-period comparison and analysis of trends in its business. Management believes that such non-GAAP measures are important in comparing current results with prior period results and are useful to investors and financial analysts in assessing the Company's operating performance. Non-GAAP financial measures are not required to be uniformly applied, are not audited and should not be considered in isolation. The non-GAAP measures give investors and financial analysts a better understanding of our net revenue and net research and development expenses without the pass-through impact of Neurocrine costs. The non-GAAP financial information presented here should be considered in conjunction with, and not as a substitute for, the financial information presented in accordance with GAAP. Investors are encouraged to review the reconciliation of these non-GAAP measures to their most directly comparable GAAP financial measures set forth below.

### Reconciliation of GAAP to Non-GAAP Measures

(in thousands)

	Three Months Ended March 31,	
	2024	2023
GAAP collaboration revenue	\$ 19,516	\$ 150,480
Revenue recognized for reimbursed research and development services (Note 1)	\$ 3,178	\$ 328
Net collaboration revenue	\$ 16,338	\$ 150,152
GAAP total research and development expenses	\$ 27,092	\$ 18,568
Expenses incurred for reimbursed research and development services (Note 1)	\$ 3,178	\$ 328
Net research and development expenses	\$ 23,914	\$ 18,240

Note 1: Under the Company's existing collaboration agreements with Neurocrine and Novartis, Neurocrine and Novartis have agreed to be responsible for all costs the Company incurs in conducting preclinical development activities for certain collaboration programs, in accordance with joint steering committee agreed upon workplans and budgets. Reimbursable research and development services performed during the period are captured within collaboration revenue and research and development expenses in the Company's consolidated statements of operations. During the three months ended March 31, 2024, we incurred \$3.2 million of reimbursable research and development services recorded within collaboration revenue and research and development expenses. During the three months ended March 31, 2023, we incurred \$0.3 million of reimbursable research and development services recorded within collaboration revenue and research and development expenses.



Source: Voyager Therapeutics, Inc.