



## Voyager Therapeutics Reports Fourth Quarter and Full Year 2023 Financial and Operating Results

02/28/24

- Company had approximately \$431 million in pro-forma cash as of December 31, 2023, adjusted for \$100 million consideration from Novartis agreements and \$100 million public offering -
- Strong cash position and anticipated milestones/reimbursements provide runway into 2027, potentially enabling the generation of clinical data from multiple programs -
- Lead development candidates selected in Friedreich's ataxia gene therapy program in collaboration with Neurocrine Biosciences, triggering \$5 million milestone payment, and in wholly-owned SOD1 ALS gene therapy program -
- Conference call at 4:30 p.m. ET today -

LEXINGTON, Mass., Feb. 28, 2024 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to advancing neurogenetic medicines, today reported fourth quarter and full year 2023 financial and operating results.

"As of December 31, 2023, Voyager had approximately \$431 million in pro-forma cash on the balance sheet, adjusted for the Novartis agreements and public offering in January 2024. We expect this funding to support the generation of clinical data across multiple programs, with the potential for significant value creation," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "We expect to advance at least four wholly-owned and partnered programs into the clinic by the end of next year. Our most advanced program, the anti-tau antibody VY-TAU01 for Alzheimer's disease, is expected to reach IND in the first half of this year, and we anticipate generating key tau PET imaging data in the second half of 2026."

### Key Milestones Achieved in Q4 2023 and Subsequent Period:

- **Strategic collaboration and capsid license agreement with Novartis:** In December 2023, Voyager entered into 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 agreed to pay 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:** In January 2024, Voyager completed an underwritten public offering of shares of its common stock (or pre-funded warrants to purchase common stock in lieu thereof) for aggregate gross proceeds of approximately \$100 million.
- **Selection of development candidate for SOD1 ALS gene therapy program:** In December 2023, Voyager announced it selected a lead development candidate for its superoxide dismutase 1 (SOD1)-mutated amyotrophic lateral sclerosis (ALS) gene therapy program. The Company expects to file an IND for this candidate in mid-2025.
- **Selection of development candidate for Neurocrine-partnered Friedreich's ataxia gene therapy program:** In February 2024, Voyager announced that the joint steering committee with its collaborator Neurocrine Biosciences selected a lead development candidate for the frataxin (FXN) gene therapy program for Friedreich's ataxia, which triggered a \$5 million milestone payment to Voyager. The companies expect the program to enter the clinic in 2025.
- **Tau silencing gene therapy program for Alzheimer's disease prioritized following in vivo proof-of-concept:** In February 2024, Voyager announced that a single intravenous administration of its tau silencing gene therapy in mice expressing human tau resulted in broad AAV distribution across multiple brain regions and dose-dependent reductions in tau messenger RNA (mRNA) levels of up to 90%, which were associated with robust reductions in human tau protein levels across the brain. The data will be presented at the upcoming 2024 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD™ 2024). Voyager has promoted the program to a prioritized program within its wholly-owned pipeline and anticipates filing an IND in 2026.

### Key Upcoming Milestones:

- **VY-TAU01 anti-tau antibody for Alzheimer's disease:** Voyager expects to file an IND in first half of 2024, initiate a Phase 1a single ascending dose study in healthy volunteers in 2024, and initiate a Phase 1b multiple ascending dose study in patients with early Alzheimer's disease in 2025. This study has the potential to generate proof-of-concept data for slowing the spread of pathological tau via tau PET imaging in 2026.
- **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, laying the foundation to potentially generate proof-of-concept based on validated biomarkers.
- **Partnered programs:** Voyager expects that its collaborative partners and licensees will submit at least two IND applications for partnered programs in Voyager's pipeline and initiate clinical development for the associated programs by the end of 2025, including the FXN gene therapy program for Friedreich's ataxia partnered with Neurocrine Biosciences.

## Fourth Quarter 2023 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$90.1 million for the fourth quarter of 2023, compared to \$(1.6) million for the same period in 2022. The increase was primarily due to \$80.0 million in collaboration revenue recognized during the fourth quarter of 2023 in connection with the 2023 Novartis collaboration agreement, \$5.3 million of revenue associated with the 2023 Neurocrine collaboration agreement, \$4.6 million of revenue associated with the 2019 Neurocrine collaboration agreement, and \$0.2 million of other collaboration revenue.
- **Net Income (Loss):** Net income was \$56.4 million for the fourth quarter of 2023, compared to net loss of \$23.6 million for the same period in 2022. The difference is primarily due to the increase in collaboration revenue discussed above.
- **R&D Expenses:** Research and development expenses were \$25.8 million for the fourth quarter of 2023, compared to \$14.6 million for the same period in 2022. The increase in R&D expenses was primarily a result of increased program-related spending, particularly manufacturing and IND-enabling studies for the anti-tau antibody program and SOD1 program, along with increased Neurocrine program support, during the fourth quarter of 2023.
- **G&A Expenses:** General and administrative expenses were \$10.2 million for the fourth quarter of 2023, compared to \$8.5 million for the same period in 2022. The increase in G&A expenses was primarily a result of \$1.9 million of business development costs related to the 2023 Novartis collaboration agreement recognized in the fourth quarter of 2023.
- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2023, were \$230.9 million. Cash position does not include proceeds received from the 2023 Novartis agreements and our underwritten public offering, both of which were received after December 31, 2023.

## Full Year 2023 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$250.0 million for the year ended December 31, 2023, compared to \$40.9 million for the same period in 2022. The increase in collaboration revenue was the result of \$79.0 million in revenue recognized during the year ended December 31, 2023, in connection with Novartis' decision to exercise two of its license options under the 2022 Novartis option and license agreement, along with the expiration of a third Novartis license option. In addition, during the year ended December 31, 2023, Voyager recognized \$80.0 million of revenue associated with the 2023 Novartis collaboration agreement, \$80.8 million of revenue associated with the 2023 Neurocrine collaboration agreement, \$9.8 million of revenue associated with the 2019 Neurocrine collaboration agreement, and \$0.4 million of other collaboration revenue. During the year ended December 31, 2022, collaboration revenue was primarily related to Pfizer's decision, as Alexion's predecessor in interest under the Alexion option and license agreement, to exercise the first license option along with the expiration of the second license option, which resulted in revenue recognized of \$40.0 million.
- **Net Income (Loss):** Net income was \$132.3 million for the year ended December 31, 2023, compared to net loss of \$46.4 million for the same period in 2022. The difference was primarily due to the revenue increases noted above.
- **R&D Expenses:** Research and development expenses were \$92.2 million for the year ended December 31, 2023, compared to \$60.8 million for the same period in 2022. The increase in R&D expenses was primarily a result of increased program-related spending, particularly manufacturing and IND-enabling studies for the anti-tau antibody program and SOD1 program, along with increased Neurocrine program support, during the 2023 period. The increase was also a result of increased compensation costs driven by headcount increases, including targeted development team hires to support the advancing pipeline, during the 2023 period.
- **G&A Expenses:** General and administrative expenses were \$35.8 million for the year ended December 31, 2023, compared to \$31.0 million for the same period in 2022. The increase in G&A expenses was primarily a result of increased compensation costs driven by headcount increases, as well as \$1.9 million of business development costs related to the 2023 Novartis agreements recognized in the fourth quarter of 2023.

## 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 including the cash received from the Novartis Collaboration and Licensing Agreement and Stock Purchase Agreement, and the completion of the public offering in January, 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 the fourth quarter and full year 2023 financial and operating results. To participate via telephone and join the call live, please register in advance here: <https://register.vevent.com/register/B1e93582a25c24475387f933ce1c5337e3>. 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 AAV capsids with robust penetration of the blood-brain barrier and enhanced central nervous system (CNS) tropism in multiple species, including non-human primates (NHPs). In preclinical studies, TRACER generated capsids have demonstrated widespread gene expression in the CNS compared to conventional AAV capsids as well as cell- and tissue-specific transduction, including to areas of the brain that have been traditionally difficult to reach, while de-targeting the liver and dorsal root ganglia. As part of its external partnership strategy, Voyager has established multiple collaboration agreements providing access to its next-generation TRACER capsids to potentially enable its partners' gene therapy programs to treat a variety of 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 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 continued development of Voyager’s technology platforms, including Voyager’s TRACER platform and its antibody screening technology; the ability to initiate and conduct preclinical studies in animal models; 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 initiation, timing, conduct and outcomes of Voyager’s preclinical and clinical studies; 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 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		Year Ended	
	December 31,		December 31,	
	2023	2022	2023	2022
<b>Statement of Operations Items:</b>				
Collaboration revenue	\$ 90,061	\$ (1,550)	\$ 250,008	\$ 40,907
Operating expenses:				
Research and development	25,756	14,551	92,172	60,764
General and administrative	10,242	8,462	35,822	30,980
Total operating expenses	35,998	23,013	127,994	91,744
Operating income (loss)	54,063	(24,563)	122,014	(50,837)
Total other income	3,154	953	11,724	4,445
Income (loss) before income taxes	57,217	(23,610)	133,738	(46,392)
Income tax provision	822	16	1,408	16
Net income (loss)	\$ 56,395	\$ (23,626)	\$ 132,330	\$ (46,408)
Net income (loss) per share, basic	\$ 1.28	\$ (0.61)	\$ 3.08	\$ (1.21)
Net income (loss) per share, diluted	1.25	(0.61)	2.97	(1.21)
Weighted-average common shares outstanding, basic	43,983,990	38,547,652	43,020,747	38,356,810
Weighted-average common shares outstanding, diluted	45,078,511	38,547,652	44,569,334	38,356,810

	December 31,	
	2023	2022
<b>Selected Balance Sheet Items</b>		
Cash, cash equivalents, and marketable securities	\$ 230,875	\$ 118,848
Total assets	\$ 351,281	\$ 159,356

Accounts payable and accrued expenses	\$	18,427	\$	10,382
Deferred revenue	\$	75,240	\$	65,827
Total stockholders' equity	\$	236,320	\$	59,020

### 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 pro-forma cash, and net collaboration revenue and net research and development expenses, the latter two of 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.

The Company's pro-forma cash as of December 31, 2023, was \$431 million which includes \$230.9 million of cash, cash equivalents, and marketable securities as of December 31, 2023, as adjusted for the gross proceeds received from the 2023 Novartis collaboration agreement and our underwritten public offering in January 2024. This does not include any costs associated with executing the Novartis collaboration or the public offering.

### Reconciliation of GAAP to Non-GAAP Measures

(in thousands)

	Three Months Ended		YTD	
	December 31,		December 31,	
	2023	2022	2023	2022
GAAP collaboration revenue	\$ 90,061	\$ (1,550)	\$ 250,008	\$ 40,907
Revenue recognized for reimbursed research and development services (Note 1)	\$ 3,052	\$ 257	\$ 10,095	\$ 836
Net collaboration revenue	\$ 87,009	\$ (1,807)	\$ 239,913	\$ 40,071
GAAP total research and development expenses	\$ 25,756	\$ 14,551	\$ 92,172	\$ 60,764
Expenses incurred for reimbursed research and development services (Note 1)	\$ 3,052	\$ 257	\$ 10,095	\$ 836
Net research and development expenses	\$ 22,704	\$ 14,294	\$ 82,077	\$ 59,928

Note 1: Under the Company's existing collaboration agreements with Neurocrine, Neurocrine has agreed to be responsible for all costs the Company incurs in conducting preclinical development activities for each Neurocrine collaboration program, 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 and twelve months ended December 31, 2023, we incurred \$3.1 million and \$10.1 million, respectively, of reimbursable research and development services recorded within collaboration revenue and research and development expenses. During the three and twelve months ended December 31, 2022, we incurred \$0.3 million and \$0.8 million, respectively, of reimbursable research and development services recorded within collaboration revenue and research and development expenses.



Source: Voyager Therapeutics, Inc.