



## Voyager Therapeutics Reports First Quarter 2023 Financial and Operating Results

May 9, 2023

*- Neurocrine Biosciences strategic collaboration and Novartis license option exercise demonstrate Voyager pipeline and platform value and strengthen balance sheet -*

*- Alzheimer's disease franchise advances: selected lead candidate for anti-tau antibody program; IND submission on track for first half of 2024; launched tau knock-down gene therapy program -*

*- Data accepted for presentation at ASGCT 2023 validate blood-brain barrier penetration and central nervous system transduction with Voyager's novel, intravenously administered capsids -*

*- Conference call at 8:30 a.m. ET today -*

CAMBRIDGE, Mass., May 09, 2023 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to breaking through barriers in gene therapy and neurology, today reported first quarter 2023 financial and operating results.

"Voyager started 2023 by securing \$175 million upfront in a strategic collaboration with Neurocrine Biosciences for our GBA1 gene therapy program and three new gene therapy programs, followed by Novartis's decision to exercise options to our novel capsids for two neurologic disease targets, triggering another \$25 million payment. These transactions have strengthened our balance sheet while maintaining long-term value and enabled us to further advance our platform and pipeline," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "We also made important progress with multiple approaches to treating Alzheimer's disease during the quarter, selecting a lead development candidate for our anti-tau antibody program and launching a new tau knockdown gene therapy program."

### Upcoming Milestones:

- **Pipeline update:**
  - Anti-tau antibody program for Alzheimer's disease: Voyager continues to expect to begin IND-enabling studies in 2023 to support an IND in the first half of 2024.
  - SOD1 gene therapy program for amyotrophic lateral sclerosis (ALS): Voyager previously announced that it expected to identify a lead development candidate for this program in the first half of 2023. The Company continues to evaluate the data from preclinical studies for this program and now expects to identify a lead development candidate in the second half of 2023.
  - Voyager and Neurocrine Biosciences continue to collaboratively advance the GBA1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases, as well as the FXN gene therapy program for Friedreich's Ataxia.
- **ASGCT 2023 Conference:** Voyager has announced seven data presentations at the American Society of Gene and Cell Therapy's (ASGCT) Annual Meeting, May 16-20, 2023, in Los Angeles, including an oral presentation titled, "Directed evolution of an AAV9 library identifies a capsid variant with enhanced brain tropism and liver de-targeting in non-human primates and mice following systemic administration."

### Key Milestones Achieved in Q1 2023 and Subsequent Period:

- **Strategic collaboration with Neurocrine Biosciences:** In January 2023, Neurocrine Biosciences, Inc. and Voyager [announced](#) the formation of a new strategic collaboration to advance Voyager's preclinical, intravenously administered GBA1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases as well as three new gene therapy programs directed to rare CNS targets. Voyager received up-front consideration of \$175 million and is eligible to receive up to \$1.5 billion in potential development milestone payments, up to \$2.7 billion in potential commercial milestone payments, tiered royalties on net sales, program funding, and an option to elect 50/50 cost- and profit-sharing in the U.S. for the GBA1 program following Phase 1 readout. The transaction closed in February 2023.
- **Novartis license of novel TRACER capsids:** In March 2023, Voyager [announced](#) that Novartis exercised its options to license novel capsids against two undisclosed neurologic disease targets. Voyager received a \$25 million option exercise payment and is eligible to receive up to \$600 million in 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.

- **Advancement of anti-tau antibody program:** In January 2023, Voyager selected a lead humanized anti-tau antibody candidate, VY-TAU01, to advance for the treatment of Alzheimer's disease. In March 2023, Voyager [presented](#) new data at the AD/PD 2023 Conference highlighting the differentiating characteristics resulting in the selection of VY-TAU01. In April 2023, Voyager received pre-IND written feedback from the FDA for VY-TAU01. Voyager continues to expect to initiate GLP toxicology studies this year to enable an IND filing in the first half of 2024.
- **Launch of new tau knockdown gene therapy early research initiative for Alzheimer's disease:** During the first quarter of 2023, Voyager announced an early research initiative investigating a gene therapy which reduces tau expression in the brain for the treatment of Alzheimer's disease. The program combines an siRNA tau knockdown payload with an intravenously delivered TRACER capsid.
- **Launch of new Huntington's disease (HD) early research initiative:** Voyager announced in January 2023 the launch of an early research initiative for HD, which combines an intravenous TRACER capsid with vectorized siRNAs to enable specific knockdown of mutant HTT and MSH3. Early data were presented at the 18th Annual Huntington's Disease Therapeutics Conference held in Zagreb, Croatia, April 24 – 27, 2023.
- **Board of Directors appointments:** In May 2023, George Scangos, Ph.D., former CEO of Exelixis, Inc.; Biogen, Inc.; and most recently Vir Biotechnology, Inc.; joined Voyager's Board of Directors. In February 2023, following the close of the collaboration with Neurocrine Biosciences, Jude Onyia, Ph.D., Chief Scientific Officer at Neurocrine Biosciences, joined the Board of Directors. In January 2023, Grace E. Colón, Ph.D., also joined the Board of Directors.

#### First Quarter 2023 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$150.5 million for the first quarter of 2023, compared to \$0.7 million for the same period in 2022. The increase was primarily due to revenue recognized during the first quarter of 2023 on the 2023 Strategic collaboration with Neurocrine Biosciences, as well as revenue recognized on the Novartis license of novel TRACER capsids.
- **Net Income (Loss):** Net income was \$124.0 million for the first quarter of 2023, compared to net loss of \$21.3 million for the same period in 2022. The difference was primarily due to the revenue increase noted above.
- **R&D Expenses:** Research and development expenses were \$18.6 million for the first quarter of 2023, compared to \$14.3 million for the same period in 2022. The increase in R&D expenses was primarily a result of increased compensation costs driven by headcount increases and program-related spend increases during the first quarter of 2023.
- **G&A Expenses:** General and administrative expenses were \$9.0 million for the first quarter of 2023, compared to \$7.7 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.
- **Cash Position:** Cash, cash equivalents and marketable securities as of March 31, 2023, were \$273.3 million. Cash position does not include \$25.0 million in option exercise payment from Novartis which was received after March 31, 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 and in future periods. As communicated previously, the Company expects its cash, cash equivalents, and marketable securities, together with the \$25.0 million options exercise payment received in April 2023 from Novartis, along with amounts expected to be received as reimbursement for development costs under the Neurocrine collaborations, to be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2025.

#### Conference Call

Voyager will host a conference call and webcast today at 8:30 a.m. ET to discuss the first quarter 2023 financial and operating results. To participate via telephone and join the call live, please register in advance [here](#). 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™ AAV 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). TRACER generated capsids have demonstrated superior and 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. Separate results have demonstrated the enhanced ability of certain capsids to target cardiac muscle and to de-target the dorsal root ganglia. Voyager is expanding its library of AAV capsids optimized to deliver diverse therapeutic payloads to address a broad range of CNS and other diseases. 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 (Nasdaq: VYGR) is a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. The potential of both disciplines has been constrained by delivery challenges; Voyager is leveraging cutting-edge expertise in capsid discovery and deep neuropharmacology capabilities to address these constraints. Voyager's TRACER AAV capsid discovery platform has generated novel capsids with

high target delivery and blood-brain barrier penetration at low doses, potentially addressing the narrow therapeutic window associated with conventional gene therapy delivery vectors. This platform is fueling alliances with Pfizer Inc., Novartis and Neurocrine Biosciences as well as multiple programs in Voyager's own pipeline. Voyager's pipeline includes wholly-owned and collaborative preclinical programs in Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Parkinson's disease, and Friedreich's Ataxia, with a focus on validated targets and biomarkers to enable a path to rapid potential proof-of-biology. 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 “may,” “might,” “will,” “would,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “target,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager's anticipated participation in future conferences, Voyager's ability to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform with increased transgene expression, increased blood-brain barrier penetration and increased biodistribution compared to conventional AAV9 and AAV5 capsids; Voyager's ability to utilize its novel proprietary capsids in its own product development programs; Voyager's ability to attract parties to license its novel proprietary capsids or to participate with Voyager in research and development collaborations utilizing its novel proprietary capsids; Voyager's ability to advance its AAV-based gene therapy programs and tau antibody program; the preclinical development of its potential development candidates; Voyager's ability to perform its obligations under its respective license option agreements with Pfizer and Novartis; Voyager's entitlement to receive option exercise, milestone and royalty-based fees from Novartis and Pfizer under the respective license option agreements; Voyager's ability to advance gene therapy product candidates under the Neurocrine collaborations into, and successfully initiate, enroll and complete, clinical trials; Voyager's entitlement to receive milestone and royalty-based fees under the Neurocrine collaborations; Voyager's ability to maintain its current partnerships and collaborations and to enter into new partnerships or collaborations; the continued service and suitability of recently appointed senior officers and members of the Board of Directors; Voyager's anticipated financial results, including the receipt by Voyager of revenues or reimbursement payments from collaboration partners; and Voyager's cash runway and Voyager's 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 studies; the possibility or the timing of the exercise of development, commercialization, license and other options under the Pfizer and Novartis license option agreements and Neurocrine collaborations; 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)

Statement of Operations Items:	Three Months Ended	
	March 31,	
	2023	2022
Collaboration revenue	\$ 150,480	\$ 658
Operating expenses:		
Research and development	18,568	14,349
General and administrative	9,028	7,659

Total operating expenses	27,596	22,008
Operating income (loss)	122,884	(21,350)
Total other income, net	1,864	31
Income (loss) before income taxes	124,748	(21,319)
Income tax provision	704	—
Net income (loss)	\$ 124,044	\$ (21,319)
Net income (loss) per share, basic	\$ 3.05	\$ (0.56)
Net income (loss) per share, diluted	\$ 2.94	\$ (0.56)
Weighted-average common shares outstanding, basic	40,632,087	38,049,430
Weighted-average common shares outstanding, diluted	42,161,326	38,049,430

	<b>March 31, 2023</b>	<b>December 31, 2022</b>
<b>Selected Balance Sheet Items</b>		
Cash, cash equivalents, and marketable securities	\$ 273,314	\$ 118,848
Total assets	\$ 336,294	\$ 159,356
Accounts payable and accrued expenses	\$ 11,337	\$ 10,382
Deferred revenue	\$ 84,552	\$ 65,827
Total stockholders' equity	\$ 216,961	\$ 59,020



Source: Voyager Therapeutics, Inc.