



Voyager Therapeutics Reports Fourth Quarter and Full Year 2022 Financial and Operating Results

03/07/23

- Transformational progress in 2022 marked by reprioritization of high-value neurology pipeline and validation of TRACER™ novel capsid platform through transactions with Pfizer and Novartis -
 - Partnering momentum continues into 2023 with \$175 million payment associated with Neurocrine Biosciences strategic collaboration followed by \$25 million payment triggered by Novartis capsid license option exercise -
 - Development candidate selected in tau antibody program for Alzheimer's disease; development candidate selection in gene therapy program for ALS expected in H1 2023; expected IND filings for both programs on track for 2024 -
 - Voyager initiates new early research initiative combining an siRNA tau knockdown payload with an intravenously delivered TRACER capsid for Alzheimer's disease -
- Conference call at 8:30 a.m. ET today -

CAMBRIDGE, Mass., March 07, 2023 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to breaking through barriers in gene therapy and neurology, today reported fourth quarter and full year 2022 financial and operating results.

"The past year has been transformational for Voyager," said Alfred W. Sandroock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "Since I became CEO of Voyager last March, we have re-prioritized our pipeline to focus on some of the most significant unmet needs in neurology, advanced our four lead programs towards the clinic, achieved breakthrough innovations in novel capsid discovery including the identification of a receptor, and entered multiple high-value partnerships. The strategic collaboration with Neurocrine that we announced in January illustrates the value of our next-generation gene therapies for CNS diseases, and this value is further evidenced by capsid license option exercises by Pfizer in October and recently by Novartis for multiple CNS targets. These partnerships strengthen our balance sheet and allow us to invest in our platform as well as existing and new neuro-genetic medicine programs."

Key Milestones Achieved in Q4 2022 and Subsequent Period:

- **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 will receive 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.
- **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 as well as three new gene therapy programs directed to rare CNS targets. Voyager received up-front consideration of \$175 million and is eligible for 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.
- **Pfizer license of novel TRACER capsid:** Voyager [announced](#) in October 2022 that Pfizer, Inc. exercised its option to license a novel capsid against an undisclosed rare neurologic disease target. Voyager received a \$10 million option exercise payment and is eligible for up to \$290 million in milestone payments plus mid- to high-single-digit tiered royalties.
- **Selection of anti-tau antibody lead candidate:** In January 2023, Voyager selected a lead humanized anti-tau antibody candidate to advance against Alzheimer's disease. The lead candidate, VY-TAU01, targets the C-terminal domain. VY-TAU01 was selected for its affinity, selectivity, and biophysical characteristics. Voyager expects to present additional data at the AD/PD 2023 Conference, March 28 – April 1, 2023. Process development and manufacturing at a CDMO have been initiated, and Voyager expects to initiate a GLP toxicology study later in 2023 to enable an IND filing in the first half of 2024.
- **Launch of updated Huntington's disease gene therapy early research initiative:** In January 2023, Voyager announced the launch of an updated early research initiative for 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 mutant HTT and MSH3.
- **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 targeting intracellular tau for the treatment of Alzheimer's disease. The program combines an siRNA tau knockdown payload with an intravenously delivered TRACER capsid.
- **ESGCT data characterize novel CNS receptor and demonstrate low-dose potential:** At the European Society of Gene & Cell Therapy (ESGCT) 29th Annual Congress in October 2022, Voyager [presented](#) data characterizing a novel cell surface receptor identified as a binding receptor for a family of Voyager's next-generation TRACER capsids. In a separate

presentation, another TRACER capsid demonstrated high levels of central nervous system (CNS) target engagement at low doses.

- **Board of Directors appointments:** In December 2022, Voyager [announced](#) the appointment of Grace E. Colón, Ph.D., to its Board of Directors, effective January 1, 2023. Dr. Colón most recently served as President, CEO and Director of the clinical-stage biotechnology company InCarda Therapeutics, Inc. In February 2023, following the close of the collaboration with Neurocrine Biosciences, Jude Onyia, Ph.D., Chief Scientific Officer at Neurocrine Biosciences, joined Voyager's Board of Directors.

Upcoming Milestones:

- **AD/PD 2023 Conference:** Voyager will present data on its GBA1 gene therapy and anti-tau antibody programs at the AD/PD 2023 Conference occurring March 28 – April 1, 2023, in Gothenburg, Sweden.
 - Oral presentation: Development of AAV-GBA1 gene replacement therapy via single-IV-delivery with a blood brain barrier penetrant AAV capsid. Charlotte Chung. April 1, 2023, 11:55 CET.
 - Poster presentation: Selection of an anti-tau antibody candidate targeting pathological tau for the treatment of Alzheimer's disease. Wencheng Liu. P0643 / #1192.
- **Pipeline progressing:** Voyager expects to identify a lead development candidate in H1 2023 for its wholly-owned SOD1 gene therapy program for amyotrophic lateral sclerosis (ALS). Voyager expects to file INDs for the SOD1 program and its wholly-owned anti-tau antibody program for Alzheimer's disease in 2024. In addition, 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.

Fourth Quarter 2022 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$(1.6) million for the fourth quarter of 2022, compared to \$28.1 million for the same period in 2021. The negative collaboration revenue during the fourth quarter of 2022 was due to an increase in the estimate of the total expected cost of Voyager's research services related to the 2019 collaboration with Neurocrine Biosciences. This increase in the estimate occurred during the fourth quarter of 2022.
- **Net (Loss) Income:** Net loss was \$23.6 million for the fourth quarter of 2022, compared to net income of \$5.7 million for the same period in 2021. The difference was primarily due to the collaboration revenue decrease.
- **R&D Expenses:** Research and development expenses were \$14.6 million for the fourth quarter of 2022, compared to \$14.0 million for the same period in 2021. The increase in R&D expenses was primarily a result of increased external research and development costs and employee-related costs in the fourth quarter of 2022 compared to the same period in 2021.
- **G&A Expenses:** General and administrative expenses were \$8.5 million for the fourth quarter of 2022, compared to \$8.4 million for the same period in 2021.
- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2022, were \$118.8 million.

Full Year 2022 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$40.9 million for 2022, compared to \$37.4 million for 2021. The increase in collaboration revenue was due to \$40.0 million in collaboration revenue recognized during the third quarter of 2022 in connection with Pfizer exercising its option to license a novel TRACER capsid, offset by a decrease in collaboration revenue from the 2019 collaboration with Neurocrine Biosciences due to a reduction of the cost of Voyager's research services.
- **Net Loss:** Net loss was \$46.4 million for 2022, compared to \$71.2 million for 2021. The difference is primarily due to the decrease in operating expenses discussed in the two bullets immediately below.
- **R&D Expenses:** Research and development expenses were \$60.8 million for 2022, compared to \$73.8 million for 2021. The decrease in R&D expenses was primarily a result of lower employee-related costs, external research and development costs, and facilities costs.
- **G&A Expenses:** General and administrative expenses were \$31.0 million for 2022, compared to \$37.2 million for 2021. The decrease in G&A expenses was primarily a result of lower employee-related costs, as well as lower facilities costs.

Financial Guidance

Voyager is committed to maintaining a strong balance sheet that supports the advancement and growth of its platform and pipeline. Following the recent transactions announced with Neurocrine Biosciences and Novartis, Voyager expects that its cash, cash equivalents, and marketable securities, together with amounts expected to be received as reimbursement for development costs under the Neurocrine collaborations, will be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2025. This could extend further based on potential future milestones from existing collaboration agreements.

Conference Call

Voyager will host a conference call and webcast today at 8:30 a.m. ET to discuss the fourth quarter 2022 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, 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, each with validated targets and biomarkers to enable a path to rapid potential proof-of-biology. For more information, visit 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; the preclinical development of its potential development candidates; Voyager's ability to develop its tau antibody program; 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 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 pre-clinical 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 Voyager's pipeline developmental 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 other 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 contractors and employees; 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
December 31,

Statement of Operations Items:

	2022	2021
Collaboration revenue	\$ (1,550)	\$ 28,073
Operating expenses:		
Research and development	14,551	14,020
General and administrative	8,462	8,351
Total operating expenses	23,013	22,371
Operating (loss) income	(24,563)	5,702
Total other income, net	953	6
(Loss) income before income taxes	(23,610)	5,708
Income tax provision	16	—
Net (loss) income	\$ (23,626)	\$ 5,708
Net (loss) income per share, basic	\$ (0.61)	\$ 0.15
Net (loss) income per share, diluted	\$ (0.61)	\$ 0.15
Weighted-average common shares outstanding, basic	38,547,652	37,804,372
Weighted-average common shares outstanding, diluted	38,547,652	37,821,320

	Year Ended December 31,	
	2022	2021
Collaboration revenue	\$ 40,907	\$ 37,415
Operating expenses:		
Research and development	60,764	73,787
General and administrative	30,980	37,246
Total operating expenses	91,744	111,033
Operating loss	(50,837)	(73,618)
Total other income, net	4,445	2,421
Loss before income taxes	(46,392)	(71,197)
Income tax provision	16	—
Net loss	\$ (46,408)	\$ (71,197)
Net loss per share, basic and diluted	\$ (1.21)	\$ (1.89)
Weighted-average common shares outstanding, basic and diluted	38,356,810	37,668,947

Selected Balance Sheet Items

	December 31, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	\$ 118,848	\$ 132,539
Total assets	\$ 159,356	\$ 193,855
Accounts payable and accrued expenses	\$ 10,382	\$ 11,524
Deferred revenue	\$ 65,827	\$ 42,096
Total stockholders' equity	\$ 59,020	\$ 95,055



Source: Voyager Therapeutics, Inc.