



Voyager Therapeutics Reports Third Quarter 2022 Financial and Operating Results

November 8, 2022

- Voyager's TRACER™ capsid platform validated by Pfizer option exercise -

- Data characterizing a novel receptor and demonstrating low-dose potential further validate TRACER -

- Pipeline programs advancing; development candidate selections for lead programs expected Q4 2022 to H1 2023 -

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

CAMBRIDGE, Mass., Nov. 08, 2022 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to breaking through barriers in gene therapy and neurology, today reported third quarter 2022 financial and operating results.

"The past few months have been characterized by increasing validation and momentum for Voyager's TRACER capsid platform," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "Pfizer's license of a novel capsid for a rare neurologic disease target reflects the potential of our platform to help overcome key limitations of current gene therapy approaches. Further, the data we presented at ESGCT characterizing a novel CNS receptor for one of our capsid families, along with data demonstrating high blood-brain barrier penetration of a capsid at doses as low as 2% of that used in current approved intravenous gene therapies, serve to increase our confidence that our preclinical capsid data has the potential to translate in human clinical trials and could be transformational for patients. Our pipeline programs for Alzheimer's disease, Parkinson's disease and ALS are progressing well, and we look forward to announcing development candidates for all three lead programs over the next several quarters, as previously guided."

Key Milestones Achieved in Q3 2022 and Subsequent Period:

- **Pfizer license of novel TRACER capsid:** Voyager [announced](#) in October that Pfizer, Inc. exercised in September its option to license a novel capsid generated from Voyager's TRACER™ capsid discovery platform to help enable a potential gene therapy program against an undisclosed rare neurologic disease target. Voyager previously received a \$30 million upfront option grant payment and, following the close of Q3 2022, received a \$10 million option exercise payment. Voyager is also eligible to receive up to \$290 million in development, regulatory, and commercial milestones, plus mid- to high-single-digit tiered royalties associated with the licensed capsid. Voyager retains global rights to the licensed capsid for use with other transgenes in its own programs and/or in business development.
- **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, Voyager [presented](#) data characterizing a novel cell surface receptor identified as a binding receptor for a family of Voyager's next-generation TRACER capsids. The newly identified receptor is highly conserved across mice, non-human primates (NHPs) and humans, increasing the potential for the associated capsid family to translate into humans. In a separate presentation, another TRACER capsid demonstrated high levels of blood-brain barrier penetration and central nervous system (CNS) target engagement when administered in the range of 2% to 20% of the dose used in currently approved intravenous gene therapies, potentially addressing the narrow therapeutic window and toxicity issues that have hampered the gene therapy field.
- **AAIC data demonstrate novel antibodies reduce spread of pathological tau:** At the Alzheimer's Association International Conference (AAIC) 2022 in August, Voyager [presented](#) preclinical data showing that both a passive antibody immunotherapy approach and a vectorized antibody gene therapy approach reduced the spread of pathological tau, which is implicated in Alzheimer's disease and other neurological diseases.
- **Expanded leadership team and Board of Directors:** In September, Voyager [announced](#) the appointment of Peter Pfreundschuh as Chief Financial Officer; Todd Carter, Ph.D., as Chief Scientific Officer; and Trista Morrison as Senior Vice President of Corporate Affairs. In July, Voyager [announced](#) the appointment of Catherine J. Mackey, Ph.D., to its Board of Directors.

Upcoming Milestones:

- **Pipeline progressing:** Voyager continues to advance its three [prioritized](#) pipeline programs: GBA1 gene therapy for Parkinson's disease, SOD1 gene therapy for amyotrophic lateral sclerosis (ALS), and a humanized anti-tau antibody for

Alzheimer's disease. The Company plans to identify lead development candidates for all three programs between Q4 2022 and H1 2023, with investigational new drug (IND) filings expected in 2024 and 2025.

- **Novartis option decision:** Voyager has an ongoing [license option agreement](#) with Novartis AG. Under this agreement, Novartis may exercise options to license novel AAV capsids generated by Voyager's TRACER capsid discovery platform for potential use with up to three undisclosed CNS targets, and Novartis may expand the license options to include TRACER capsids for two additional targets. The Novartis license decision and the decision on the expansion of the license option are expected by March 2023.

Third Quarter 2022 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$41.1 million for the third quarter of 2022, compared to \$1.5 million for the same period in 2021. The increase in collaboration revenue was due primarily to \$40.0 million in collaboration revenue recognized during the third quarter in connection with Pfizer exercising its option to license a novel capsid generated from the TRACER capsid discovery platform.
- **Net Income (Loss):** Net income was \$17.6 million for the third quarter of 2022, compared to a net loss of \$25.1 million for the same period of 2021. The difference is primarily due to the increase in collaboration revenue discussed above.
- **R&D Expenses:** Research and development expenses were \$19.3 million for the third quarter of 2022, compared to \$17.9 million for the same period in 2021. The increase in R&D expenses was primarily a result of increased external research and development costs, offset by lower employee-related costs and lower facilities costs.
- **G&A Expenses:** General and administrative expenses were \$7.3 million for the third quarter of 2022, compared to \$8.7 million for the same period in 2021. The decrease in G&A expenses was primarily a result of lower employee-related costs, as well as lower facilities costs.
- **Cash Position:** Cash, cash equivalents and marketable securities as of September 30, 2022, were \$131.6 million. Pfizer's \$10 million TRACER capsid option exercise payment, received in October 2022, is not included in this number.

Year-to-Date 2022 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$42.5 million for the year-to-date 2022 period, compared to \$9.3 million for the same period in 2021. The increase in collaboration revenue was primarily due to \$40.0 million in collaboration revenue recognized during the third quarter in connection with Pfizer exercising its option to license a novel TRACER capsid.
- **Net Loss:** Net loss was \$22.8 million for the year-to-date 2022 period, compared to \$76.9 million for the same period of 2021. The difference is primarily due to the increase in collaboration revenue discussed above, along with the decrease in operating expenses discussed below.
- **R&D Expenses:** Research and development expenses were \$46.2 million for the year-to-date 2022 period, compared to \$59.8 million for the same period in 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 \$22.5 million for the year-to-date 2022 period, compared to \$28.9 million for the same period in 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 expects that its cash, cash equivalents, and marketable securities, together with amounts expected to be received as reimbursement for development costs under the Neurocrine collaboration, will be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2024. Beyond Neurocrine, the Company's runway could improve further based on potential future milestones in 2023 from existing collaboration agreements.

Conference Call

Voyager will host a conference call and webcast today at 8:30 a.m. ET to discuss the third quarter 2022 financial and operating results. To participate via telephone and join the call live, please register in advance here: <https://register.vevent.com/register/Bla813418ea1c148dda525a8560a51e1ca>. 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 preclinical programs in Parkinson's disease, Alzheimer's disease, and amyotrophic lateral sclerosis (ALS), 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 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 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 a member 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 severity and length of the COVID-19 health crisis; 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, as updated by its subsequent filings 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:

Collaboration revenue
Operating expenses:
Research and development
General and administrative
Total operating expenses
Operating income (loss)
Total other income, net
Net income (loss)
Net income (loss) per share, basic
Net income (loss) per share, diluted
Weighted-average common shares outstanding, basic
Weighted-average common shares outstanding, diluted

Three Months Ended September 30,		
	2022	2021
	\$ 41,086	\$ 1,482
	19,337	17,914
	7,307	8,714
	26,644	26,628
	14,442	(25,146)
	3,182	9
	\$ 17,624	\$ (25,137)
	\$ 0.46	\$ (0.67)
	\$ 0.45	\$ (0.67)
	38,507,542	37,780,547
	39,570,394	37,780,547

Collaboration revenue
Operating expenses:
Research and development
General and administrative
Total operating expenses
Operating loss
Total other income, net
Net loss
Net loss per share, basic and diluted
Weighted-average common shares outstanding, basic and diluted

Nine Months Ended September 30,		
	2022	2021
	\$ 42,457	\$ 9,342
	46,213	59,767
	22,518	28,895
	68,731	88,662
	(26,724)	(79,320)
	3,492	2,414
	\$ (22,782)	\$ (76,906)
	\$ (0.59)	\$ (2.04)
	38,292,497	37,623,309

Selected Balance Sheet Items

Cash, cash equivalents, and marketable securities
Total assets
Accounts payable and accrued expenses
Deferred revenue
Total stockholders' equity

	September 30,	December 31,
	2022	2021
	\$ 131,638	\$ 132,539
	\$ 182,480	\$ 193,855
	\$ 13,866	\$ 11,524
	\$ 64,021	\$ 42,096
	\$ 79,823	\$ 95,055



Source: Voyager Therapeutics, Inc.