



Voyager Therapeutics Reports Robust Preclinical Activity in Tau Silencing Gene Therapy Program for Alzheimer's Disease and Advances Program into Late Research

02/20/24

- Additional new data demonstrate VY-TAU01, Voyager's lead antibody targeting pathological tau, was well-tolerated and demonstrated favorable pharmacokinetics in non-human primates (NHPs) -

- Data to be presented at the 2024 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD™ 2024) -

LEXINGTON, Mass., Feb. 20, 2024 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to advancing neurogenetic medicines, today announced new data from its two preclinical programs targeting pathological tau for the treatment of Alzheimer's disease. Data on VY-TAU01, Voyager's lead anti-tau antibody candidate, and on Voyager's tau silencing gene therapy program will be presented at the upcoming 2024 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD™ 2024), taking place March 5-9, 2024, in Lisbon, Portugal.

Tau Silencing Gene Therapy Program:

- Intravenous administration of BBB-penetrant AAV containing primary artificial microRNA targeting tau reduces tau broadly and robustly in hTau mouse brain. Wencheng Liu, Ph.D., Poster P0783/#2852.

The data demonstrate that a single intravenous (IV) administration of one of Voyager's tau silencing gene therapy candidates 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. Voyager's tau silencing gene therapy program combines vectorized tau-targeted siRNA with a proprietary, blood-brain barrier (BBB)-penetrant capsid derived from the Company's TRACER™ discovery platform. Based on these data, Voyager has advanced this program into late research and expects to file an investigational new drug (IND) application in 2026.

VY-TAU01 Anti-Tau Antibody:

- Pharmacokinetics and tolerability of VY-TAU01, an anti-tau antibody for the treatment of Alzheimer's disease, in P301S mouse and nonhuman primate. Wencheng Liu, Ph.D., Poster P0795/#1303.

Voyager also will report new preclinical data demonstrating that VY-TAU01, the Company's lead antibody targeting pathological tau, and Ab-01, its murine surrogate, were well-tolerated and showed favorable pharmacokinetic profiles following IV administration in NHPs and P301S transgenic mice expressing pathological human tau. Voyager continues to anticipate an IND filing for VY-TAU01 in the first half of 2024.

"The Voyager team is increasingly excited about the potential for treatments targeting pathological tau to play an important role in improving clinical outcomes for patients suffering with Alzheimer's disease," said Todd Carter, Ph.D., Chief Scientific Officer of Voyager Therapeutics. "Recent third-party data with tau-targeted investigational therapies have demonstrated reduced spread of pathological tau, as measured by tau PET imaging, and favorable trends in cognition. We look forward to continuing to advance our two programs targeting tau, and observing anticipated data read-outs from others in this space that will play an important role in further validating this target."

About Alzheimer's Disease

Alzheimer's disease is a progressive neurodegenerative disease estimated to affect 6 million people in the U.S.ⁱ and up to 416 million people globallyⁱⁱ. The disease causes memory loss and may escalate to decreased independence, communication challenges, behavioral disorders such as paranoia and anxiety, and lack of physical controlⁱⁱⁱ. In 2023, the total cost of caring for people living with Alzheimer's and other dementias in the U.S. is estimated at \$345 billion^{iv}.

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.

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 “will,” “expect,” “continue,” “anticipate,” or “potential,” and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager’s ability to advance its tau silencing gene therapy program and anti-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 potential for treatments targeting pathological tau to improve clinical outcomes for patients suffering with Alzheimer’s disease; and the potential for additional third-party data read-outs to provide further validation for pathological tau as a target 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 Voyager’s receipt of program reimbursement, development or commercialization milestones, option exercise, and other payments under Voyager’s current 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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ⁱ Alzheimer’s Association. 2023 Alzheimer’s Facts and Figures. Available at: <https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf>. Accessed February 15, 2024.

ⁱⁱ Gustavsson A, Norton N, Fast T, et al. Global estimates on the number of persons across the Alzheimer’s disease continuum. *Alzheimer’s Dement*. 2023; 19: 658–670. doi: 10.1002/alz.12694.

ⁱⁱⁱ Penn Medicine. The 7 Stages of Alzheimer’s Disease. Available at: <https://www.pennmedicine.org/updates/blogs/neuroscience-blog/2019/november/stages-of-alzheimers>. Accessed February 15, 2024.

^{iv} USAgainstAlzheimer’s. The Alzheimer’s Disease Crisis – By the Numbers. Available at: [The Alzheimer’s Disease Crisis – By the Numbers | UsAgainstAlzheimer’s \(usagainstalzheimer.org\)](https://usaagainstalzheimer.org). Accessed: February 15, 2024.



Source: Voyager Therapeutics, Inc.