



Voyager Therapeutics Announces First Participants Dosed in Single Ascending Dose Trial of VY-TAU01 for the Treatment of Alzheimer's Disease

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LEXINGTON, Mass., May 16, 2024 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to advancing neurogenetic medicines, today announced that the first participants were dosed in a Phase 1a single ascending dose (SAD) trial of VY-TAU01, an investigational anti-tau antibody developed to inhibit the spread of pathological tau in Alzheimer's disease.

The objective of the randomized, double-blind, placebo-controlled, SAD trial is to evaluate the safety and pharmacokinetics of VY-TAU01 in healthy adult volunteers. The study is being conducted at a single site in the United States and is expected to enroll approximately 48 patients in multiple cohorts. Voyager anticipates that the data from the SAD trial will inform the design of a Phase 1b multiple ascending dose (MAD) trial in patients with early Alzheimer's disease, which Voyager expects to initiate in 2025. The MAD study has the potential to generate initial tau PET imaging data in the second half of 2026, which may indicate if VY-TAU01 can slow the spread of pathological tau in the brain.

"The initiation of clinical development of VY-TAU01 for the treatment of Alzheimer's disease is an important milestone for Voyager; it demonstrates the executional abilities of our neurology drug development team, which will be central to our advancement of three wholly-owned and partnered neurology gene therapies towards IND filings next year," said Toby Ferguson, M.D., Ph.D., Chief Medical Officer of Voyager Therapeutics. "Alzheimer's disease remains an area with tremendous unmet patient need, despite recent advances. We are encouraged by our preclinical data demonstrating the ability of VY-TAU01 to significantly slow tau spreading, and we look forward to evaluating the therapeutic potential of VY-TAU01 in the clinic."

About VY-TAU01

VY-TAU01 is an IV-administered, recombinant, humanized IgG4 monoclonal antibody developed to inhibit the spread of pathological tau, which is closely correlated with disease progression and cognitive decline in Alzheimer's disease. In contrast to previous N-terminal directed anti-tau antibodies that did not show efficacy in clinical studies, VY-TAU01 targets a distinct C-terminal epitope of tau and has demonstrated robust in vivo inhibition of the spread of pathological tau in a preclinical model. Additional preclinical studies have demonstrated that VY-TAU01 was well-tolerated and demonstrated a favorable pharmacokinetic profile following IV administration.

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 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 "potential," "anticipate," "expect," "will," "may," 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 the generation of clinical data; the potential for clinical data from the SAD trial to inform the design of the Voyager's anticipated MAD trial; and Voyager's ability to advance gene therapy product candidates under its partnered programs 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.

Contacts

Trista Morrison, NACD.DC, tmorrison@vvygr.com

Investors: Adam Bero, Ph.D., abero@kendallir.com

Media: Brooke Shenkin, brooke@scientpr.com

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.