# Voyager Presents Robust Preclinical Data from Tau Targeting Gene Therapy and Antibody Programs at AD/PD™ 2025

03/31/25

*- Single IV administration of tau silencing gene therapy VY1706 significantly reduced tau mRNA and protein levels, with broad brain distribution and liver de-targeting, in NHP study -*

*- Preclinical murine data strengthen case for specifically targeting pathologic forms of tau with the clinical-stage anti-tau antibody VY7523 -*

*- Voyager to host live webcast on April 7 recapping key AD/PD™ 2025 data -*

LEXINGTON, Mass., March 31, 2025 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to leveraging genetics to treat neurological diseases, today reported new data from its two preclinical programs targeting tau for the treatment of Alzheimer's disease (AD). Data on tau silencing gene therapy VY1706 and anti-tau antibody VY7523 will be presented at the upcoming 2025 International Conference on Alzheimer's and Parkinson's Diseases and Related Neurological Disorders (AD/PD™ 2025), taking place April 1-5, 2025, in Vienna. Additionally, Voyager announced it will host a live webcast recapping key AD/PD™ 2025 data at 4:30 p.m. ET on Monday, April 7, 2025.

"Emerging data continue to strengthen our conviction that tau is the next critical target in Alzheimer's disease, which supports Voyager's advancement of both antibody and gene therapy approaches," said Toby Ferguson, M.D., Ph.D., Chief Medical Officer of Voyager Therapeutics. "When I look across the design of these two molecules, the preclinical data each has generated, and the emerging clinical profile for our antibody, I believe our anti-tau antibody and gene therapy each have the potential to be transformative for patients."

**VY1706 tau silencing gene therapy:**

CROSS-SPECIES BBB-PENETRANT IV-DELIVERED AAV GENE THERAPY PROVIDES BROAD AND ROBUST CNS TAU LOWERING IN TAUOPATHY MOUSE AND NON-HUMAN PRIMATE. Wencheng Liu, Ph.D., ID:419.

New preclinical data from a non-human primate (NHP) study show that a single, intravenous (IV) 1.3E13 vg/kg dose of Voyager's tau silencing gene therapy VY1706 resulted in dose-dependent knockdown of tau mRNA (44% to 73%) and tau protein (27% to 55%). Results were sustained up to three months following dosing, and VY1706 was well-tolerated at both the 5-week and 11-week timepoints assessed in the study. Broad distribution across the brain was achieved, including throughout areas impacted by AD such as the hippocampus, entorhinal cortex, temporal cortex, and frontal cortex regions, while the liver was de-targeted by approximately 30-fold compared to wild type AAV9. VY1706 is in Investigational New Drug (IND)-enabling studies with an IND filing expected in 2026.

**VY7523 anti-tau antibody:**

DISCRIMINATION OF ANTI-TAU ANTIBODIES TARGETING DIFFERENT TAU EPITOPES BY A P301S MOUSE HIPPOCAMPAL SEEDING MODEL OF TAUOPATHY. Wencheng Liu, Ph.D., ID:1403.

New preclinical data show that the murine version of Voyager's VY7523 demonstrates selectivity for binding pathologic tau tangles. Additionally, Voyager assessed several anti-tau antibodies using a P301S mouse hippocampal seeding model of tau spread. Antibodies that bind the N-terminal of tau and previously failed to achieve their primary endpoints in clinical studies also failed to significantly reduce tau spread in the model. Conversely, the murine version of Voyager's VY7523, which targets the C-terminal of tau, reduced tau spread in the model, as did a third-party mid-domain antibody that has been shown to reduce tau accumulation in a clinical trial. Voyager is currently assessing VY7523 in a multiple ascending dose trial in patients with early AD and expects initial tau positron emission tomography (PET) imaging data in the second half of 2026.

The posters can be accessed on Voyager's website at: https://www.voyagertherapeutics.com/science-publications/.

Dr. Ferguson will be speaking on two panels at AD/PD™ 2025:

- Challenges and Opportunities for Anti-Tau Therapies in Clinical Trials – How Can We Make Hopes Come True? Saturday, April 5, 2025, at 2:10 p.m. in Hall A
- Drug Development and Biomarkers in Rare CNS Diseases (ALS, FTD): From Basics to Approval – How to Define Success? Saturday, April 5, 2025, at 4:40 p.m. in Hall A

**Live Webcast**
Voyager will host a live conference call and webcast at 4:30 p.m. ET on Monday, April 7, 2025, to discuss data from AD/PD™ 2025. A live webcast of the call will be available on the Investors section of the Voyager website at https://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 VY1706**
VY1706 is an IV-administered gene therapy for Alzheimer's disease that combines a potent siRNA construct to decrease the expression of tau with an IV-delivered, blood-brain barrier-penetrant TRACER™ capsid. In preclinical non-human primate (NHP) studies, a single IV administration of VY1706 was well-tolerated and resulted in dose-dependent knockdown of tau mRNA (44% to 73%) and tau protein (27% to 55%) broadly across the brain. Voyager anticipates filing an investigational new drug (IND) application with the U.S. Food and Drug Administration (FDA) and a clinical trial application (CTA) with Health Canada in 2026.

**About VY7523**
VY7523 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. VY7523 is selective for pathological tau and targets a specific C-terminal epitope of tau. In preclinical in vivo studies, the murine version of VY7523 inhibited seeding and spread of pathological tau by approximately 70%. In a Phase 1, single ascending dose (SAD) clinical trial in healthy volunteers, VY7523 demonstrated a safety, tolerability, immunogenicity, and pharmacokinetic profile warranting advancement. Voyager is currently assessing VY7523 in a multiple ascending dose trial in patients with early Alzheimer's disease and expects initial tau PET imaging data in the second half of 2026.

**About Alzheimer's Disease**
Alzheimer's disease is a progressive neurodegenerative disease estimated to affect 7 million people in the U.S. [i] and up to 416 million people globally[ii]. The disease causes memory loss and may escalate to decreased independence, communication challenges, behavioral disorders such as paranoia and anxiety, and lack of physical control[iii]. In 2023, the total cost of caring for people living with Alzheimer's and other dementias in the U.S. was 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, Friedreich's ataxia, Parkinson's disease, amyotrophic lateral sclerosis (ALS), 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; and Neurocrine Biosciences, Inc. For more information, visit 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 "will," "expect," "believe," "anticipate," "potential," "may," or "continue," 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 the IND and CTA filings, the initiation of clinical trials, clinical trial enrollment, and the generation of clinical data; Voyager's plans to present scientific data at future conferences; the commercial potential for VY7523 and VY1706; the importance of tau as a target for the treatment of AD; and the potential for third-party clinical data to inform Voyager's clinical development plans 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 and 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 expectations and decisions of regulatory authorities; the timing, initiation, conduct and outcomes of Voyager's preclinical and clinical studies; the availability of data from clinical trials; the availability or commercial potential of product candidates under collaborations; the success of Voyager's product candidates; the willingness and ability of Voyager's collaboration partners to meet obligations under collaboration agreements with Voyager; the continued development of Voyager's technology platforms, including Voyager's TRACER platform and its antibody screening technology; Voyager's scientific approach and program development progress, and the restricted supply of critical research components; 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 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 success of programs controlled by third-party collaboration partners in which Voyager retains a financial interest; the ability to attract and retain talented directors, employees, and contractors; and the sufficiency of Voyager's 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@vygr.com
Investors: Sarah McCabe, smccabe@jpa.com
Media: Brooke Shenkin, brooke@scientpr.com

References:

i. Alzheimer's Association. 2024 Alzheimer's Facts and Figures. Available at: https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf. Accessed January 9, 2025.

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 January 9, 2025.

iv. USAgainstAlzheimer's . The Alzheimer's Disease Crisis – By the Numbers. Available at: The Alzheimer's Disease Crisis – By the Numbers | UsAgainstAlzheimer's (usagainstalzheimers.org). Accessed: January 9, 2025.

Source: Voyager Therapeutics, Inc.