Voyager Receives FDA IND Clearance for VY1706, First Gene Therapy Approach to Reducing Tau Production in the Brain for Alzheimer's Disease

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- Dosing of adults with early Alzheimer's disease in clinical trial expected H2 2026 -

- Comprehensive preclinical program showed compelling pharmacology and safety profile for VY1706 -

- VY1706 uses an IV-delivered, brain-targeted capsid to reduce intracellular and extracellular tau -

LEXINGTON, Mass., June 01, 2026 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to leveraging genetics to treat neurological diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for VY1706, the Company's investigational gene therapy targeting intracellular and extracellular tau for Alzheimer's disease (AD). The IND clearance enables initiation of a clinical trial of VY1706 in adults with early AD; dosing is expected to begin in the second half of the year.

"The IND clearance for VY1706 is the first for a tau-targeted gene therapy and follows a comprehensive preclinical program demonstrating a compelling pharmacology and safety profile," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "Recent third-party data continue to suggest that tau is the next critical target in Alzheimer's disease, and that reducing tau production holds promise. We view VY1706 as leading the next generation of tau targeting treatments; it is designed to durably reduce tau protein levels in key brain regions following a single IV administration."

The core of VY1706 is a potent, vectorized siRNA that targets MAPT mRNA to decrease levels of both intracellular and extracellular tau in the brain. This core is encapsulated in a Voyager TRACER™ AAV capsid that leverages ALPL, a well-conserved, novel receptor identified by Voyager, to deliver the siRNA into the brain following a one-time, intravenous (IV) dose. The efficacy and safety of VY1706 have been assessed in a comprehensive preclinical program spanning multiple species. VY1706 has been demonstrated to reduce tau protein up to 75% in key brain regions relevant to AD and to de-target the liver, a source of adverse events associated with other systemically administered gene therapies.

Clinical Trial Design

Voyager is initiating a multi-site, open-label, dose-escalation clinical trial of VY1706 administered as a one-time IV dose to adult participants with early AD who have evidence of tau pathology in the brain, confirmed by PET imaging. The study will enroll up to 18 patients across three cohorts, with the highest dose not exceeding 5E13 vg/kg, the top dose tested in the good laboratory practice (GLP) toxicology study. The trial's primary endpoint is to evaluate the safety and tolerability of VY1706. Secondary endpoints will assess VY1706's effect on tau biology, including changes in cerebrospinal fluid (CSF) biomarkers of tau, as well as changes in tau pathology measured by tau PET imaging.

Preclinical Data Supporting VY1706

Three-month GLP toxicology data for VY1706 demonstrated a favorable tolerability profile with no adverse clinical pathology or histopathological findings up to the highest dose tested (5E13 vg/kg) at 13 weeks following a single IV dose in non-human primates (NHPs). Treatment with VY1706 resulted in dose-dependent reductions of up to 51-75% MAPT mRNA and 48-64% tau protein in key brain regions implicated in AD, such as the entorhinal cortex, frontal cortex, temporal cortex, and hippocampus.[1] These findings were consistent with findings from a previous NHP study.[2] Further, murine studies have demonstrated robust and dose-dependent reductions in pathological tau protein levels following a single IV dose in the P301S mouse tauopathy model.[3]

About VY1706

VY1706 is an investigational gene therapy for Alzheimer's disease (AD) that targets tau, a protein associated with neurodegeneration and cognitive decline in AD. The core of VY1706 is a potent, vectorized siRNA that targets MAPT mRNA to decrease levels of both intracellular and extracellular tau in the brain. This core is encapsulated in a Voyager TRACER™ AAV capsid that leverages ALPL, a well-conserved, novel receptor identified by Voyager, to deliver the siRNA into the brain following a one-time, intravenous (IV) dose. The efficacy and safety of VY1706 have been assessed in a comprehensive preclinical program spanning multiple species. VY1706 has been demonstrated to reduce tau protein up to 75% in key brain regions relevant to AD and to de-target the liver, a source of adverse events associated with other systemically administered gene therapies. Voyager is initiating a clinical trial of VY1706 administered as a one-time IV dose to adults with early AD.

About Alzheimer's Disease (AD)

AD is a fatal neurodegenerative disease characterized by severe memory impairment and loss of the ability to carry out everyday tasks as the disease progresses[4]; it impacts approximately 7.2 million people in the U.S.[5], and 32 million people globally are estimated to have AD dementia[6]. After disease onset, patients experience a profound decline in quality of life, and death occurs within an average of four to eight years of diagnosis[5]. Tau pathology correlates strongly with neurodegeneration and cognitive decline in AD[7], and a high unmet medical need remains for disease-modifying therapies that address tau pathology and further slow disease progression. In 2025, the total cost of caring for people living with Alzheimer's and other dementias in the U.S. was estimated at $384 billion[5].

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 novel AAV capsids to enable gene therapy. Voyager has leveraged TRACER to create multiple families of novel capsids that, following intravenous delivery in preclinical studies, harness the extensive vasculature of the central nervous

system (CNS) to cross the blood-brain barrier and transduce a broad range of CNS regions and cell types. In cross-species preclinical studies (rodents and multiple non-human primate species), intravenous delivery of TRACER-generated capsids resulted in widespread payload expression across the CNS at relatively low doses, enabling selection of multiple development candidates in Voyager's wholly-owned and partnered gene therapy programs for neurologic 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, 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.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, including, without limitation, implied and express statements about Voyager's beliefs and expectations regarding Voyager's advancement of the VY1706 program, including the timing and achievement of clinical development milestones such as Voyager's intentions to initiate clinical trials, clinical trial enrollment, and achievement of first-in-human dosing in AD in the second half of 2026; the therapeutic potential, clinical benefit, safety and pharmacological effect of VY1706; the ability of clinical trials to demonstrate safety and efficacy of Voyager's product candidates such as VY1706; the potential role of tau in the treatment of AD, including the potential clinical benefit of tau reduction; the potential for Voyager's novel TRACER capsids to achieve desired results in humans; Voyager's ability to execute across its pipeline and platforms; and the mission and goals for Voyager's business. The use of words such as "may," "will," "might," "would," "could," "should," "expect," "plan," "anticipate," "believe," "potential," "intend," "seek," "predict," "estimate," "project," "target," or "continue" and other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

All forward-looking statements are based on management's current estimates and assumptions and are subject to a number of risks, uncertainties, and important factors that may cause actual results to differ materially from any forward-looking statements in this press release. Factors include, among others, the risks and uncertainties inherent in the development of product candidates, including the initiation, enrollment, timing, cost, progress, and results of Voyager's planned and future clinical trials; expectations and decisions of regulatory authorities; Voyager's ability to replicate positive results from earlier preclinical studies or clinical trials in current or future clinical trials; potential adverse events Voyager may encounter that could negatively impact development; outcomes of third-party preclinical studies and clinical trials that could impact Voyager's development plans; Voyager's ability to demonstrate that current or future product candidates are safe and effective for their proposed indications; Voyager's scientific approach and continued development of its technology platforms, including the TRACER and non-viral discovery platforms; the development by third parties of capsid or non-viral identification platforms that may be competitive to its platforms and programs; Voyager's ability to create and protect its intellectual property rights; the progress and success of programs under current or future collaboration and license agreements; the sufficiency of Voyager's cash resources to fund its operations and pursue its corporate objectives; and technical and other unexpected hurdles in the development, manufacture and supply of Voyager's product candidates, may delay its timing, change its plans, increase its costs, or otherwise negatively impact its business or the sufficiency of its cash resources to fund operations.

These risks and uncertainties 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 this press release is as of today's date, 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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[1] Arora et al. ASGCT 2026. [2] Sivasankaran et al. ADPD 2026. [3] Bao et al. ASGCT 2025. [4] Masters CL, Bateman R, Blennow K, et al. Alzheimer's disease. Nat Rev Dis Primers. 2015;1:15056. [5] 2025 Alzheimer's disease facts and figures. Alzheimers Dement. 2025;21(5). [6] Gustavsson A, Norton N, Fast T, et al. Global estimates on the number of persons across the Alzheimer's disease continuum. Alzheimers Dement. 2023;19:658-70. [7] Braak H, Braak E. Neuropathological staging of Alzheimer-related changes. Acta Neuropathol. 1991;82(4):239-59.