Voyager ASGCT Late Breaker: Single IV Dose of VY1706 Well Tolerated, Reduced Tau in 3-Month GLP Toxicology Data; Clinical Trial in Alzheimer's Disease Expected H2 2026

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- VY1706 IND application process on track for Q2 2026; clinical entry expected H2 2026 -

- Data from eight ASGCT presentations highlight Voyager's continued innovation in gene therapy -

LEXINGTON, Mass., May 13, 2026 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to leveraging genetics to treat neurological diseases, today presented three-month good laboratory practice (GLP) toxicology data for VY1706, the Company's investigational tau silencing gene therapy for Alzheimer's disease (AD), in a late-breaking presentation at the American Society of Gene & Cell Therapy's (ASGCT) 2026 Annual Meeting in Boston, May 11-15, 2026. Data presented showed that VY1706 was well tolerated, with no adverse clinical pathology or histopathological findings up to the highest dose tested (5E13 vg/kg), and reduced tau protein up to 64% in key brain regions of non-human primates (NHPs) at 13 weeks following a single IV dose.

Voyager's U.S. Food and Drug Administration (FDA) investigational new drug (IND) application process for VY1706 is on track for Q2 2026 to support projected first-in-human dosing in AD patients in H2 2026.

"These latest 3-month GLP toxicology data with tau silencing gene therapy VY1706 are consistent with the robust preclinical data package we have established to date, and we look forward to advancing into clinical trials for Alzheimer's disease later this year," said Todd Carter, Ph.D., Chief Scientific Officer of Voyager Therapeutics. "VY1706 has the potential to be the first gene therapy approach to targeting tau, and we believe the ability to knock down intracellular and extracellular tau with a one-time, IV dose could be transformative for patients living with Alzheimer's disease."

Late-Breaking Oral Presentation
Intravenous delivery of VY1706, a CNS penetrant AAV gene therapy for Alzheimer's disease, demonstrates compelling pharmacology and safety in a 3-month GLP toxicology study in NHPs (#163).

- VY1706 delivered a potent vectorized MAPT siRNA with broad central nervous system (CNS) distribution via a single IV dose, using ALPL as its primary blood-brain barrier (BBB) receptor.
- 3-month GLP toxicology data for VY1706 demonstrated a favorable tolerability profile with no adverse findings up to the highest dose tested (5E13 vg/kg).
- Treatment with VY1706 resulted in dose-dependent reductions of up to 51-75% MAPT mRNA and 48-64% tau protein in key brain regions at 13 weeks in NHPs.

Data from Voyager's seven additional oral and poster presentations at ASGCT demonstrate continued capsid innovation via muscular and neuromuscular targeting, immune evasion, and manufacturability.

Oral Presentation
Directed evolution of muscular and neuromuscular capsid variants in both mice and non-human primates (#422).

- Voyager leveraged its TRACER ™ platform to develop novel AAV9-based capsids with significantly increased muscle tropism over AAV9 in both mice and NHPs.
- In mice, the top muscle-targeted capsid achieved 100X increased expression over AAV9 in skeletal muscle and 10X increased expression in heart muscle.
- In NHPs, the top muscle-targeted capsid achieved 25-30X increased expression over AAV9 in skeletal muscle and 13X increased expression in heart muscle.
- Voyager generated neuromuscular capsids with features of both muscle and brain capsids, achieving comparable transduction levels to top muscle and brain capsids in NHPs while demonstrating significantly reduced liver exposure relative to AAV9.

Alzheimer's Disease Targets
Intravenous delivery of a bi-functional AAV gene therapy to reduce endogenous ApoE4 and express ApoE2 in ApoE4 humanized mice (#1460).

- A novel BBB-penetrant capsid delivering a bi-functional payload significantly reduced endogenous ApoE4 while achieving expression of ApoE2 at physiological levels following single dose IV-delivery in murine studies.

Leveraging TRACER Beyond the CNS and Reducing Immunogenicity
Engineering an AAV9-derived muscle-tropic capsid to evade pre-existing human neutralizing antibodies (#1028).

- A next-generation "stealth" capsid was able to evade pre-existing neutralizing antibodies while retaining muscle tropism, potentially increasing the number of patients eligible to receive muscle-targeted AAV gene therapies.

Leveraging artificial intelligence to design AAV mutant capsids optimized for antibody evasion (#2027).

- Novel AAV capsid variants, generated using advanced AI models, showed improvements in antibody evasion, while maintaining favorable manufacturing and CNS-transduction properties.

Enhancing Developability and Manufacturing of Capsids
Exploiting an AAV capsid specific receptor to develop stable cell lines for transduction based assays for gene therapies (#3148).

- Cell lines overexpressing a receptor associated with Voyager's TRACER ™ capsids overcome the challenge of lower transduction efficiency observed for engineered capsids in cell-based assays.

Evaluating affinity chromatography media for capture of novel blood-brain-barrier penetrant AAV capsids (#3161).

- Analyses of variables in affinity chromatography provide insights into optimizing scale-up to support manufacturing of Voyager's novel capsids.

Optimized transfection platform with improved productivity and transgene packaging for scalable rAAV production (#3139).

- Optimization of manufacturing processes delivers a higher product quality profile and improved yields to enable scale-up for GLP toxicology and good manufacturing practice (GMP) production of Voyager's novel capsids.

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 belief and expectations regarding Voyager's advancement of its AAV-based gene therapy programs, including expectations for and timing with regards to achievement of preclinical and clinical development milestones for VY1706 such as the IND application process in the second quarter of 2026 and achievement of first-in-human dosing in AD patients in the second half of 2026, pending successful IND clearance; the preclinical data for and potential safety and pharmacological effect of VY1706; the potential for VY1706 to be the first gene therapy approach to targeting tau and its transformative potential in treating AD; the importance of tau as a target for the treatment of AD; the potential for Voyager's novel TRACER capsids to achieve desired results in humans, including to expand potential indications beyond the CNS via muscular and neuromuscular targeting and to expand the eligible patient population via immune evasion; the ability of Voyager's improvements in manufacturing to enable increased yields and large-scale development of AAV gene therapies; Voyager's ability to execute across our pipeline and platforms, including continued innovation in capsid discovery; and the mission, goals and value drivers for our 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, 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 our technology platforms, including the TRACER and non-viral discovery platforms; the development by third parties of capsid identification platforms that may be competitive to our platforms and programs; Voyager's ability to create and protect our 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 our operations and pursue our corporate objectives; and technical and other unexpected hurdles in the development, manufacture and supply of our product candidates, may delay our timing, change our plans, increase our costs, or otherwise negatively impact our business or the sufficiency of our 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 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: Adam Silverstein, adam@scientpr.com

Source: Voyager Therapeutics, Inc.