Voyager Adds Fourth Wholly-Owned Alzheimer's Disease Program to Pipeline, Complementing Existing Tau and Amyloid Assets with New APOE Approach

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- New program combines IV-delivered TRACER™ capsid with bifunctional payload to silence APOE in carriers of the high-risk APOE4 variant while delivering the protective APOE2 variant -

- Voyager's wholly-owned Alzheimer's disease franchise now includes clinical-stage anti-tau antibody VY7523 as well as gene therapies targeting tau, amyloid, and APOE -

LEXINGTON, Mass., July 16, 2025 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to leveraging genetics to treat neurological diseases, today continued the expansion of its Alzheimer's disease (AD) franchise with the introduction of a wholly-owned program that modulates the expression of apolipoprotein E (APOE), the strongest genetic risk factor for AD.[i] The program uses a proprietary intravenous (IV)-delivered, blood-brain barrier (BBB)-penetrant TRACER capsid to deliver a bifunctional payload that is designed to decrease expression of APOE in carriers of the variant APOE4, while delivering the variant APOE2. APOE4 has been strongly linked with a higher risk of developing AD, with almost all APOE4 homozygotes exhibiting AD pathology[i], while APOE2 has been associated with a lower risk of developing AD.

In preclinical studies, a single IV injection of a TRACER capsid carrying the single bifunctional vector resulted in significant reductions of endogenous APOE4 in key AD-relevant brain regions of APOE4 knock-in mice, while significantly increasing expression of the APOE2 isoform to maintain overall APOE levels. Voyager anticipates presenting early data on this program at an upcoming scientific meeting in 2025.

"The Voyager team is leveraging our deep expertise in Alzheimer's disease biology and drug development to advance multiple programs against what we believe to be the three most-promising targets: tau, amyloid, and APOE," said Alfred W. Sandrock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "We believe each of these approaches will have an important role to play in the treatment of Alzheimer's disease, particularly as the field begins to understand how best to sequence and combine treatments to improve outcomes for patients. We look forward to near-term data on some of these targets expected from third parties, which we expect will continue to inform our Alzheimer's disease franchise and approach."

Voyager's AD franchise is now comprised of four wholly-owned assets:

- VY7523, a pathologic-specific anti-tau antibody, which is being evaluated in a multiple ascending dose (MAD) clinical trial in AD patients, with initial tau positron emission tomography (PET) data expected in the second half of 2026.
- VY1706, an IV-delivered tau silencing gene therapy that has shown up to 73% knockdown of tau mRNA in non-human primates following a single IV dose and is advancing towards IND in 2026.
- A vectorized anti-Aβ antibody gene therapy, which demonstrated over 15-fold greater brain-to-plasma ratio after a single IV dose compared to a passively administered antibody over 4 weeks in a murine model, as presented at the American Society of Gene & Cell Therapy's (ASGCT) 28th Annual Meeting.
- The APOE gene therapy program, which is designed to knock down APOE in APOE4 carriers while delivering APOE2 and maintaining total APOE levels.

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

For example, all statements Voyager makes regarding Voyager’s continued expansion of its AD franchise by advancing multiple programs against tau, amyloid and APOE to treat AD and the potential to sequence and combine such treatments to improve therapeutic outcomes for patients; 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 filings, the initiation of clinical trials, clinical trial enrollment, and the generation of clinical data; Voyager’s ability to advance preclinical programs (i) against ApoE using a single IV-injected TRACER capsid to deliver a bifunctional payload to reduce endogenous ApoE4, as well as (ii) against amyloid by its vectorized anti-Ab antibody gene therapy. 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 non-viral discovery platform; Voyager’s scientific approach and program development progress, and the restricted supply and increased costs 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 and non-viral discovery platform, the ligands identified by the platform, and development and clinical 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.

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[i] Fortea, J., Pegueroles, J., Alcolea, D. *et al. APOE4* homozygosity represents a distinct genetic form of Alzheimer’s disease. *Nat Med* **30**, 1284–1291 (2024). https://doi.org/10.1038/s41591-024-02931-w

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