Voyager Therapeutics Presents New Data Supporting Tau Antibody Program for Alzheimer's Disease and GBA1 Gene Therapy Program for Parkinson's Disease at the AD/PD™ Conference

March 28, 2023

CAMBRIDGE, Mass., March 28, 2023 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to breaking through barriers in gene therapy and neurology, today presented new data from its anti-tau antibody program for Alzheimer's disease (AD) and from its GBA1 gene therapy program for Parkinson's disease (PD) and other GBA1-mediated diseases at the AD/PD™ Conference in Gothenburg, Sweden.

"Voyager continues to advance multiple programs towards the clinic. For our anti-tau antibody program for Alzheimer's disease, these new preclinical data support our recent selection of VY-TAU01 as the lead development candidate, with an IND filing expected in the first half of 2024," said Todd Carter, Ph.D., Chief Scientific Officer at Voyager. "For our GBA1 gene therapy program with Neurocrine for Parkinson's disease, these new data from a GBA1 loss of function mouse model show effects of three GBA1 gene therapy candidates on multiple endpoints."

Selection of an Anti-tau Antibody Candidate Targeting Pathological Tau for the Treatment of Alzheimer's Disease (Wencheng Liu, Poster P0643 / #1192)

- Tau pathology, and specifically the neuron-to-neuron transmission of pathologic tau, is hypothesized to contribute to cognitive decline and neurodegeneration in AD. Voyager is advancing an anti-tau antibody that blocks pathologic tau spreading for the treatment of AD.
- Through multiple immunization campaigns that resulted in a pool of 728 candidates, Voyager identified four novel antibodies that met criteria regarding selectivity, affinity, functional inhibition of pathologic tau in vitro and in vivo, and developability. Three of these antibodies target the same epitope on the C-terminal domain of tau, while one antibody targets the mid-domain; all four are differentiated from N-terminal targeted approaches that have previously failed in clinical trials. All four Voyager antibodies demonstrated robust preclinical efficacy in blocking the spread of pathological tau in a P301S seeding-propagation tauopathy mouse model. Ab01 demonstrated superior efficacy (>70% reduction in tau pathology, as presented at AAIC 2022), while N-terminal antibodies typically lack efficacy in this model.
- Ab01 was humanized. After analysis of multiple humanized Ab01 variants, Voyager selected VY-TAU01 as the development candidate based on a number of developability characteristics, including favorable affinity, activity, specificity, stability, and immunogenicity.

Development of AAV-GBA1 gene replacement therapy via single-IV-delivery with a blood brain barrier penetrant AAV capsid (Charlotte Chung, April 1, 2023, 11:55 CET, #1696)

- The GBA1 gene encodes the lysosomal enzyme β-glucocerebrosidase (GCase). Mutations in GBA1 decrease GCase activity and cause increased levels of substrate; these mutations are one of the greatest genetic risk factors for PD. Voyager and Neurocrine Biosciences are advancing a GBA1 gene therapy leveraging a novel, intravenously administered, blood-brain-barrier-penetrant TRACER™ capsid for the treatment of PD.
- Voyager has previously demonstrated that this GBA1 gene therapy approach delivered therapeutically-relevant levels of GCase to the brain in mouse efficacy models, following a single intravenous dose using a blood-brain-barrier penetrant capsid.
- These new data from additional mouse efficacy studies showed that all three candidates demonstrated significant improvement in several efficacy biomarkers.

About Voyager Therapeutics

Voyager Therapeutics (Nasdaq: VYGR) is a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. The potential of both disciplines has been constrained by delivery challenges; Voyager is leveraging cutting-edge expertise in capsid discovery and deep neuropharmacology capabilities to address these constraints. Voyager's TRACER AAV capsid discovery platform has generated novel capsids with high target delivery and blood-brain barrier penetration at low doses, potentially addressing the narrow therapeutic window associated with conventional gene therapy delivery vectors. This platform is fueling alliances with Pfizer Inc., Novartis and Neurocrine Biosciences as well as multiple programs in Voyager's own pipeline. Voyager's pipeline includes wholly-owned and collaborative preclinical programs in Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Parkinson's disease, and Friedreich's Ataxia, each with validated targets and biomarkers to enable a path to rapid potential proof-of-biology. For more information, visit 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 “may,” “might,” “will,” “would,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “target,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding preclinical data for its antibody programs and AAV-based gene therapy programs, including the presentation of preclinical data at the 2023 AD/PD Conference; Voyager’s ability to continue to identify and develop anti-tau antibodies; Voyager’s ability to develop its tau antibody program; Voyager’s ability to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform with increased transgene expression, increased blood-brain barrier penetration and increased biodistribution compared to conventional AAV9 and AAV5 capsids; Voyager’s ability to utilize its novel proprietary capsids in its own product development programs; Voyager’s ability to advance its AAV-based gene therapy programs; the preclinical development of its potential development candidates; and Voyager’s ability to utilize its TRACER capsids and antibodies in its product development 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 pre-clinical 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 Voyager’s pipeline developmental programs; the initiation, timing, conduct and outcomes of Voyager’s preclinical studies; the ability to attract and retain talented contractors and employees; 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.

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Source: Voyager Therapeutics, Inc.