Voyager Therapeutics Receives FDA Clearance of IND Application for Gene Therapy Candidate VY-HTT01 for Treatment of Huntington's Disease

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One-Time Gene Therapy to be Evaluated in Patients with Early Huntington's Disease

Initiation of VYTAL Phase 1/2 Clinical Trial at Multiple Sites Expected this Year

CAMBRIDGE, Mass., April 26, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe neurological diseases, today announced the U.S. Food and Drug Administration (FDA) has removed its clinical hold on the company's Investigational New Drug (IND) application for VY-HTT01, a gene therapy candidate for the treatment of Huntington's disease (HD), and confirmed that the company may proceed with its planned Phase 1/2 clinical trial. The decision was made following a comprehensive review of the Chemistry, Manufacturing and Controls information previously submitted to the FDA. Voyager plans to initiate VYTAL, a Phase 1/2 clinical trial of VY-HTT01, this year.

"The decision by the FDA regarding our IND application for VY-HTT01 for Huntington's disease represents an important milestone for Voyager and is the result of years of commitment to developing an impactful new therapy to address this devastating disease," said Andre Turenne, President and CEO of Voyager.

Currently there are no disease-modifying therapies approved for the treatment of Huntington's disease, a fatal, inherited neurodegenerative disease caused by toxic gain-of-function mutations in the *huntingtin*, or HTT, gene. HD affects over 30,000 people in the U.S. alone, with symptom onset commonly appearing between the ages of 30 to 50. The disease is marked by progressive decline of motor and cognitive functions and a range of behavioral and psychiatric disturbances.

VY-HTT01 is a gene therapy designed to reduce the expression of *huntingtin*, thereby altering disease progression. VY-HTT01 is comprised of an adeno-associated virus capsid (AAV1) and a proprietary transgene that harnesses the canonical RNA interference pathway to selectively knock down levels of HTT mRNA. Preclinical data in non-human primates demonstrated robust and durable reduction of HTT mRNA and protein and widespread distribution of VY-HTT01 across the striatum and cortex, which are core areas of disease pathology.

"Our investigational gene therapy has been designed to achieve broad knockdown of HTT mRNA throughout the brain via a one-time MRI-guided neurosurgical delivery," said Omar Khwaja, CMO and Head of Research and Development of Voyager. "We are thrilled to be collaborating with leading experts in Huntington's disease and neurosurgical delivery of gene therapies as we begin the planned clinical evaluation of our promising candidate."

Voyager's VYTAL Phase 1/2 clinical trial is a dose escalation study to evaluate the safety and tolerability of VY-HTT01 in patients with early manifest Huntington's disease. Secondary endpoints include disease biomarkers and clinical outcome measures.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases. Voyager is committed to advancing the field of AAV gene therapy through innovation and investment in vector engineering and optimization, manufacturing, and dosing and delivery techniques. Voyager's wholly owned and partnered pipeline focuses on severe neurological diseases for which effective new therapies are needed, including Parkinson's disease, Huntington's disease, Friedreich's ataxia, and other severe neurological diseases. For more information on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com or follow @VoyagerTx on Twitter and LinkedIn.

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," "undoubtedly," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements. For example, all statements Voyager makes regarding the initiation, timing, progress, activities and goals of the VYTAL clinical trial of VY-HTT01, the ability of VY-HTT01 to achieve broad knock down of HTT mRNA throughout the brain, the collaboration by Voyager with leading Huntington's disease and neurosurgical experts, and Voyager's intentions and programs for the development of a gene therapy for Huntington's disease are forward looking statements.

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 ability to arrange for the initiation of the VYTAL clinical trial at clinical sites, including the ability to receive the review by and approval of Institutional Review Boards and other necessary institutional approvals at proposed clinical sites, the ability to recruit and engage researchers, physicians and patients to participate in the VYTAL clinical trial, the ability to agree to terms, and to sign and implement clinical trial agreements and associated agreements with VYTAL clinical trial collaborators, the ability to identify Huntington patients who meet the eligibility requirements for the VYTAL clinical trial, and the ability for Voyager

to conduct a clinical trial in a timely manner under pending Covid-19 pandemic protocols. These statements are also subject to a number of material risks and uncertainties that are described in Voyager's 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.

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