Voyager Therapeutics Selects Lead Clinical Candidate for Huntington's Disease

June 1, 2017

VY-HTT01 Selection Based on Robust Knockdown of Huntingtin in Preclinical Studies Program On Track for IND Filing in 2018

CAMBRIDGE, Mass., June 01, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe neurological diseases, today announced the selection of VY-HTT01, a clinical candidate for the treatment of Huntington's disease. Huntington's disease is a fatal, inherited neurodegenerative disease that results in the progressive decline of motor and cognitive functions caused by an expansion mutation in the *huntingtin*, or HTT, gene. VY-HTT01 is composed of an adeno-associated virus (AAV) capsid and proprietary transgene that harnesses the RNA interference pathway to selectively knock down the production of HTT messenger RNA (mRNA). Direct delivery of VY-HTT01 to the brain with a one-time administration could potentially slow or halt the progression of this uniformly fatal disease. Preclinical pharmacology and toxicology studies are now underway with VY-HTT01 to support filing of an investigational new drug (IND) application in 2018.

"We systematically and thoroughly optimized the AAV capsid and transgene before selecting VY-HTT01 as the lead clinical candidate for Huntington's disease with scientists at Sanofi Genzyme, as part of our research alliance," said Dinah Sah, Ph.D., chief scientific officer at Voyager. "In preclinical models, a single administration of VY-HTT01 was well-tolerated and resulted in robust and widespread knockdown of HTT messenger RNA in disease-relevant regions of the non-human primate central nervous system. The extent of HTT mRNA suppression (greater than 50%) and high precision and efficiency of primary microRNA processing in these preclinical studies supported the selection of VY-HTT01 as our lead clinical candidate. Pre-IND safety studies are now underway in order to advance VY-HTT01 to Phase 1 clinical trials. In addition, as part of our candidate selection process, we carried out extensive optimization of the vector genome resulting in a configuration that provided excellent yield and genome integrity for manufacturing scale-up of VY-HTT01 using Voyager's baculovirus AAV manufacturing process in insect-derived cells."

"CHDI Foundation is delighted to be collaborating with Voyager, a leader in developing gene therapy programs for severe neurological diseases," said Robi Blumenstein, president of CHDI Management, Inc. "The selection of VY-HTT01 as their lead clinical candidate is a significant milestone that brings us an important step closer to a clinical trial of a therapeutic for Huntington's disease."

About Huntington's Disease

Huntington's disease is a fatal, inherited neurodegenerative disease that results in progressive motor, neuropsychiatric, and cognitive impairment. A genetic mutation comprising an expanded trinucleotide (CAG) repeat in the *huntingtin* gene is causal for the disease and drives pathogenesis through a toxic gain-of-function. Approximately 30,000 individuals in the U.S. are diagnosed with the disease and approximately 200,000 individuals in the U.S. are at risk of inheriting the disease, according to the Huntington's Disease Society of America. Each child of a parent with a mutation in the *huntingtin* gene has a 50% chance of inheriting the disease, with symptoms usually appearing between the ages of 30 to 50. Quality of life is substantially affected and death occurs within 15 to 25 years of overt clinical onset. There are currently no therapeutics approved that slow the progression of Huntington's disease and only one drug, tetrabenazine, has been approved for the treatment of the specific motor symptoms of Huntington's disease.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company 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. The company's pipeline focuses on severe neurological diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of ALS, Huntington's disease, Friedreich's ataxia, frontotemporal dementia, Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, and the University of Massachusetts Medical School. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics is headquartered in Cambridge, Massachusetts. For more information, please visit www.voyagertherapeutics.com. Follow Voyager on LinkedIn.

About CHDI

CHDI Foundation, Inc. (CHDI) is a privately-funded nonprofit biomedical research organization that is exclusively dedicated to rapidly developing therapies that slow the progression of Huntington's disease. As a collaborative enabler, CHDI seeks to bring the right partners together to identify and address critical scientific issues and move drug candidates to clinical evaluation as quickly as possible. CHDI scientists work closely with a network of more than 700 researchers in academic and industrial laboratories around the world in the pursuit of these novel therapies, providing strategic scientific direction to ensure that our common goals remain in focus. More information about CHDI can be found at www.chdifoundation.org.

In September 2016, Voyager announced a research collaboration with CHDI to advance Voyager's VY-HTT01 program building upon a previous collaboration between CHDI and Sanofi Genzyme that includes funding from CHDI to help support preparation for and filing of an investigational new drug application, as well as completion of a Phase 1 clinical trial. CHDI will be reimbursed for its support of Voyager's program upon VY-HTT01 achieving certain commercial milestones.

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 law. The use of words such as "may," "might," "will," "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 and reporting of results of its preclinical programs and clinical trials and its research and development programs, its ability to advance its AAV-based gene therapies, such as VY-HTT01, into, and successfully complete, clinical trials, its ability to continue to develop its product engine, its ability to add new programs to its pipeline, its ability to develop manufacturing capability for its products, its ability to enter into new partnerships or collaborations, and the timing or likelihood of its submissions and filings with and approvals from the FDA and other regulatory authorities, are forward looking. All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes 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. 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 and Quarterly Report on 10-Q filed with the Securities and Exchange Commission, as updated by its future filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Investor Relations: Matt Osborne Head of Investor Relations & Corporate Communications 857-259-5353 mosborne@vygr.com Media: Katie Engleman Pure Communications, Inc. 910-509-3977 Katie@purecommunicationsinc.com

Voyager Therapeutics