Voyager Therapeutics Announces Lead Clinical Candidate Selection for Monogenic Form of Amyotrophic Lateral Sclerosis (ALS)

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VY-SOD101 Selected Based on Robust Delivery and Knock-Down of Toxic SOD1 in Preclinical Models

Program On Track for IND filing during 4Q:17

CAMBRIDGE, Mass., Feb. 13, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the central nervous system (CNS), today announced the selection of VY-SOD101, a clinical candidate for the treatment of ALS due to mutations in the superoxide dismutase 1 gene (SOD1). Multiple studies have demonstrated that mutant SOD1 is toxic to motor neurons, and leads to their progressive loss. VY-SOD101 is composed of a proprietary adeno-associated virus (AAV) capsid and transgene with a micro RNA (miRNA) expression cassette that harnesses the RNAi pathway to selectively silence, or knock-down, the production of SOD1 messenger RNA. With a single intrathecal (IT) injection, VY-SOD101 has the potential to durably reduce the levels of toxic mutant SOD1 protein in the CNS to slow the progression of disease. Preclinical pharmacology and toxicology studies are now underway to support filing of an investigational new drug (IND) application for VY-SOD101 during the fourth quarter of 2017.

"To select an AAV gene therapy candidate for a particular disease, Voyager considers a number of features of the overall candidate profile including optimization of the AAV vector capsid, the transgene, and the dosing paradigm," said Dinah Sah, Ph.D, senior vice president of neuroscience at Voyager. "The VY-SOD101 clinical candidate was selected after screening a series of capsids, microRNA expression cassettes and encoded payloads. Multiple rounds of optimization resulted in a candidate that is potent and selective. In addition, many construct configurations were evaluated toward the identification of one which would provide excellent yield and genome integrity for manufacturing scale-up in Voyager's baculovirus AAV manufacturing system in insect-derived cells. Preclinical data in large mammals demonstrated that a single IT administration resulted in robust knock-down of SOD1 in motor neurons, and based on these results, we are excited to progress VY-SOD101 closer towards the clinic and to those living with this devastating disease."

"Voyager's pipeline targeting severe, often fatal, diseases of the CNS is rapidly progressing," said Steven Paul, M.D., president and chief executive officer at Voyager. "One of the core competencies of our company is vector optimization, as Dinah and her team have demonstrated with the selection of VY-SOD101 for ALS. Importantly, vector delivery and manufacturing at scale are additional core competencies, which will undoubtedly facilitate the clinical development of VY-SOD101. During 2017, we remain committed to progressing lead candidate selections for our pipeline programs that are close behind VY-SOD101, including VY-HTT01 for Huntington's disease, and VY-FXN01 for Friedreich's ataxia."

About Amyotrophic Lateral Sclerosis

Amyotropic Lateral Sclerosis (ALS) is a rare, rapidly progressive, fatal disease characterized by the degeneration of nerve cells in the spinal cord and brain resulting in severe muscle atrophy with loss of the ability to walk and speak, and premature death. The median survival is approximately three years, and 90 percent of people with ALS die within five years of symptom onset.[1] ALS affects approximately 20,000 people in the U.S., with less than 10,000 new cases identified each year reflecting a high rate of mortality and short survival, relative to other diseases with similar incidences.[2]

Patients with ALS typically develop weakness in one body region (upper or lower limb or bulbar) and then develop symptoms and signs of progressive dysfunction of motor neurons. The majority of ALS cases occur sporadically and with unknown cause, but in approximately 10 percent of patients, the cause is familial and can be linked to an identifiable genetic defect. An estimated 20 percent of familial cases can be attributed to mutations in SOD1, the first mutant gene discovered to be causal for the development of ALS, through a toxic gain of function mechanism leading to motor neuron pathogenesis.[3] Riluzole is the only drug approved by the U.S. Food and Drug Administration for the treatment of ALS. In controlled trials, Riluzole delayed the time to onset of tracheostomy or death by approximately two to three months but did not improve muscle strength or neurological function.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the CNS. 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 CNS diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of ALS, Friedreich's ataxia, Huntington's disease, 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.

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 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 expected cash, cash equivalents and marketable securities at the end of a fiscal year and anticipation for how long expected cash, cash equivalents and marketable securities will last, and the timing or likelihood of its regulatory filings and approvals, 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 Quarterly Report on Form 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.

[1] Sorenson EJ, et al. (2002) Neurology 59:280-282

[2] www.alsa.org

[3] Rosen D, et al. (1993) Nature 362:59-62

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