Voyager Therapeutics Announces Additional Data at the American Society of Gene and Cell Therapy 2018 Annual Meeting

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Voyager's gene therapy vector for Friedreich's ataxia prevents central and peripheral disease progression in preclinical model out to one year after a single administration

Durable safety data generated with Voyager's novel AAV gene therapy capsids along with widespread gene transfer to the brain and spinal cord of non-human primates after a single intravenous administration

CAMBRIDGE, Mass., May 18, 2018 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases today announced additional data presentations at the American Society of Gene and Cell Therapy (ASGCT) taking place May 16-19, 2018, in Chicago, Ill. This evening at ASGCT, Voyager presented results for its gene therapy program for Friedreich's ataxia as well as data related to Voyager's novel adeno-associated virus (AAV) capsid optimization efforts.

"We continue to advance our preclinical gene therapy programs, which include optimizing the capsids, or the outer shell of the gene therapy vector, for effective delivery to the central nervous system after intravenous administration," said Dinah Sah, Ph.D., Voyager's chief scientific officer. "Data presented this evening at ASGCT describe the exciting progress with capsids that cross the blood-brain barrier and enhance transduction of the brain and spinal cord of non-human primates and in a preclinical model of Friedreich's ataxia. The data also demonstrate good tolerability of these capsids in over 20 non-human primates up to three months based on in-life observations, clinical chemistry and histopathology."

Data presentations at this year's ASGCT meeting can be found on the Events and Presentations page of the Investors and Media section of Voyager's corporate website at www.voyagertherapeutics.com

Oral presentation for novel gene therapy capsids:

Oral Presentation Title: "Safety and Increased Transduction Efficiency in the Adult Nonhuman Primate Central Nervous System with Intravenous Delivery of Two Novel Adeno-Associated Virus Capsids" Abstract O661

> Intravenous dosing of AAV gene therapy vectors in adult large mammals has resulted in limited transfer across the blood-brain barrier to achieve gene expression in the central nervous system (CNS). Data previously presented from Voyager's studies in adult non-human primates demonstrated that novel AAV gene therapy capsids facilitated widespread, enhanced gene transfer to the brain and spinal cord after a single, intravenous (IV) administration. One month after dosing of a transgene encoding both a therapeutic protein (frataxin) and a reporter (HA-tag) to facilitate molecular and immunohistochemical measurements, substantial levels of frataxin-HA were expressed in the CNS of the non-human primate, including motor neurons throughout the length of the spinal cord, and neurons in the brain stem, motor cortex, substantia nigra, thalamus and cerebellar dentate nucleus.
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> At this year's ASGCT meeting, one and three-month safety data on novel capsids were presented demonstrating good tolerability, based on in-life observations, clinical chemistry and histopathology in 26 non-human primates. Voyager continues to conduct additional studies in non-human primates with these and other novel AAV capsids to optimize delivery of genes to regions critical for the treatment of amyotrophic lateral sclerosis (ALS), Parkinson's disease and Friedreich's ataxia (FA), as well as other severe neurological diseases.

Oral presentation for Voyager's preclinical Friedreich's ataxia program:

Oral Presentation Title: "Rescue of Central and Peripheral Neurological Phenotype in a Mouse Model of Friedreich's Ataxia by Intravenous Delivery of AAV Frataxin with a Novel Capsid" Abstract O672

> FA is a severe, inherited neurological disease caused by mutations in the frataxin (FXN) gene leading to decreased expression of FXN, which results in severe sensory impairment, progressive loss of the ability to walk, generalized weakness, and loss of sensation, as well as severe and potentially fatal cardiomyopathy. Data presented at this year's ASGCT meeting demonstrated in a transgenic mouse model of FA, that one-time, post-symptomatic IV dosing of Voyager's vector composed of a novel AAV capsid and a frataxin transgene durably improved motor function and rescued the FA phenotype based on multiple functional tests of sensory and motor behavior. These functional tests included the notched-bar and rotarod motor deficit tests that require sensory and motor coordination to perform. In both tests, Voyager's frataxin gene therapy vector demonstrated dose-dependent and durable responses for more than 10 months after a single administration, preventing central and peripheral disease progression.
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> In the rotarod test at 15.5 weeks of age, and at the highest dose, the mouse receiving Voyager's frataxin gene therapy vector maintained out to one year the time spent on the rotarod at approximately 200 seconds compared to a decline to 40 seconds in the diseased (knockout) mouse. For the notched-bar traversing test, at the highest dose, Voyager's frataxin gene therapy vector prevented progression of ataxia, reducing the number of foot slips out to one year when compared to those observed in the knockout mouse. Additional preclinical studies are underway at Voyager including steps to optimize a lead clinical candidate for the treatment of FA.

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 pipeline focuses on severe neurological diseases in need of effective new therapies,

including Parkinson's disease, a monogenic form of ALS called SOD1, Huntington's disease, Friedreich's ataxia, neurodegenerative diseases related to defective or excess aggregation of tau protein in the brain including Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, AbbVie, 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.

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 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 initiate, enroll and complete, clinical trials, the potential clinical utility of its product candidates, its ability to continue to develop its gene therapy platform, its ability to develop manufacturing capability for its products and successfully transition its manufacturing process, its ability to perform under existing collaborations with, among others, Sanofi Genzyme and AbbVie and to add new programs to its pipeline, its ability to enter into new partnerships or collaborations, 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. Such risks and uncertainties include, among others, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; the continued development of the gene therapy platform; Voyager's scientific approach and general development progress; and the availability or commercial potential of Voyager's product candidates. 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, as updated by its subsequent 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.

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