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

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VY-SOD101 achieves meaningful suppression of disease-causing gene of ALS in large mammals after a one-time administration

Results during the second half of 2018 from further delivery optimization efforts with VY-SOD101 and VY-HTT01 for Huntington's disease expected to support IND filings in 2019

CAMBRIDGE, Mass., May 17, 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 new data presentations at the American Society of Gene and Cell Therapy (ASGCT) taking place May 16-19, 2018, in Chicago, Ill. Voyager presented results for VY-SOD101, which targets the superoxide dismutase 1 gene (SOD1), the first mutant gene discovered to be causal for the development of amyotrophic lateral sclerosis (ALS). The results demonstrated that a one-time administration of VY-SOD101 lowered SOD1 mRNA levels by 78% in the spinal cord motor neurons of non-human primates. Additional new data at this year's ASGCT meeting included tolerability data in non-human primates for VY-HTT01 for Huntington's disease, along with previous data with VY-HTT01 that demonstrated a 54% suppression of huntingtin (HTT) mRNA in the non-human primate putamen after a single administration. Presentations at ASGCT also included comparability data between Voyager's baculovirus/Sf9 and HEK293 triple transfection manufacturing systems.

"Voyager's multiple preclinical programs continue to progress and this year's ASGCT meeting revealed data demonstrating robust lowering, or knockdown, of disease-causing genes for our ALS and Huntington's disease programs and good tolerability of these approaches," said Dinah Sah, Ph.D., Voyager's chief scientific officer. "Importantly, we showed knockdown in comprehensive sample sets in these studies in non-human primates as well as precision and efficiency of pri-miRNA processing using deep sequencing. These results establish the potential of our gene therapy constructs to suppress the genetic cause of disease as we move forward into planned clinical trials. Efforts to further optimize delivery for our ALS and Huntington's disease programs are underway, and we plan to provide these results during the second half of this year."

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

Voyager investor/analyst breakfast event at ASGCT:

Voyager's senior management team will review data being presented at the ASGCT meeting during the following webcasted event:

Date/time: Friday, May 18, 2018, 7:30 a.m. CDT
Location: Joliet Room, 3rd Floor, Hilton Chicago Hotel, 720 S. Michigan Ave., Chicago, Ill.

The live call may be accessed by dialing (877) 851-3834 for domestic callers, or +1 (631) 291-4595 for international callers, and referencing conference ID number 4685038. A live audio webcast of the conference call will be available online from the Investors & Media section of Voyager's website at www.voyagertherapeutics.com.

Poster presentation for VY-SOD101 for a genetic cause of ALS:

Title: "Selection of an AAV Gene Therapy Targeting SOD1 for the Treatment of SOD1-ALS" Poster P555

ALS is a 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. Mutations in the SOD1 gene lead to motor neuron loss through a toxic gain of function mechanism and accounts for two to three percent of ALS cases worldwide. Intrathecal (IT) administration of AAV vectors in preclinical models of ALS has transferred genes to the spinal cord, providing less exposure to peripheral tissues and reducing the impact of pre-existing immunity compared with systemic dosing. With an IT injection, VY-SOD101 has the potential to durably reduce the levels of toxic mutant SOD1 protein in the spinal cord to slow the progression of disease.

Previously, Voyager presented data evaluating IT delivery of an AAV gene therapy vector targeting SOD1 using RNAi for the treatment of canine degenerative myelopathy, a naturally-occurring disease of companion dogs that is similar to some forms of human ALS, including the SOD1 form of disease. A single IT administration of VY-SOD101 resulted in 74% and 41% knockdown of SOD1 mRNA in the dorsal root ganglia and spinal cord, respectively, and was well-tolerated in canines.

Data at this year's ASGCT meeting included knockdown and tolerability assessments of VY-SOD101 in non-human primates four weeks after IT administration. VY-SOD101 lowered SOD1 mRNA by 78% in spinal cord motor neurons as measured by RT-qPCR and was well-tolerated with observed no vector-related effects on body weight, clinical signs, clinical pathology, cerebrospinal fluid chemistry or total cell counts, or histopathology of the spinal cord or liver. Further optimization of delivery in large animal models is ongoing with results expected in the second half of this year.

Oral presentation for VY-HTT01 for Huntington's disease:

Title: "Pharmacology and Safety of VY-HTT01, an AAV miRNA Gene Therapy Targeting Huntingtin for the Treatment of Huntington's Disease" Abstract O28

Voyager previously selected VY-HTT01 as 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 HTT gene. Data previously presented on VY-HTT01 showed a 54% suppression of HTT mRNA in the non-human primate putamen after a single administration, as measured quantitatively by branched DNA.

Data at this year's ASGCT meeting included tolerability assessments five weeks after VY-HTT01 administration. VY-HTT01 was well-tolerated in non-human primates with no observed associated changes in clinical pathology or adverse histopathological findings at the site of administration within the putamen, with no clinical symptoms, supporting the selection of this vector as the lead clinical candidate. Further optimization of delivery is ongoing, data from which will be presented during the second half of this year. Preclinical pharmacology and toxicology studies are underway with VY-HTT01 to support filing of an investigational new drug (IND) application.

Poster presentation describes comparability data between Voyager's baculovirus/Sf9 system and HEK triple transfection manufacturing system:

Title: "Biophysical and In Vitro Comparability Analysis of an AAV Vector Produced by the Baculovirus/Sf9 System and HEK Triple Transfection System" Abstract P100

Voyager's manufacturing platform utilizes a baculovirus/Sf9 cell production process that enables the production of AAV vectors at clinical and commercial scale, with the potential for increased yields over traditional production processes. A recently cleared IND application for Voyager's VY-AADC for Parkinson's disease included data demonstrating comparability between VY-AADC produced under good manufacturing practice (GMP) using Voyager's baculovirus/Sf9 manufacturing process and VY-AADC produced using a mammalian cell system consisting of triple-transfection of human embryonic kidney (HEK293) cells. Data at this year's ASGCT described the comparability of AAV vectors produced with baculovirus/Sf9 cells demonstrating similar analytical characteristics and *in vitro* and *in vivo* pharmacological activity when compared to AAV vectors produced using HEK293 cells. Other data demonstrated that the baculovirus/Sf9 production system can be used to produce AAV vectors that are indistinguishable with respect to bio-distribution, potency and expression in vivo compared to AAV vectors produced in mammalian cells.

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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