Voyager Therapeutics Highlights Lead Program VY-AADC for Advanced Parkinson's Disease, AAV Novel Capsid and Delivery Optimization Efforts, and Pipeline Programs at its R&D Day

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CAMBRIDGE, Mass., Nov. 16, 2017 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for severe neurological diseases, today at its R&D Day highlighted recent progress and plans for VY-AADC for advanced Parkinson's disease, and progress with testing novel adeno-associated virus (AAV) capsids and delivery optimization efforts, along with its ALS, Huntington's disease, Friedreich's ataxia, anti-Tau antibody and severe chronic pain preclinical programs.

"Results from our primary market research of physicians and patients presented today at our R&D day reflect the excitement surrounding our VY-AADC program and its one-time treatment, unique mechanism of action, and potential for marked and durable improvements in patients' motor function," said Steve Paul, M.D., president and chief executive officer of Voyager Therapeutics. "In addition, based on these attributes, neurologists indicated an interest to recommend VY-AADC to patients even before they reach an advanced stage of disease. Our global, pivotal program for VY-AADC in advanced Parkinson's disease remain on track, and we are excited to consider this and other areas of Parkinson's disease with this program. Also at the R&D day, we described our innovative work optimizing capsid and vector delivery and moving only highly-targeted and differentiated candidates into the clinic."

Senior executives from Voyager Therapeutics' scientific, clinical and management team, provided the following highlights at the R&D day:

- Results from primary market research indicating a strong interest among neurologists, movement disorder specialists, neurosurgeons and patients for VY-AADC for advanced Parkinson's disease. The primary market research was conducted following the recent Phase 1b results with VY-AADC that demonstrated durable, dose-dependent and time-dependent improvements across multiple measures of patients' motor function after a one-time administration, and with meaningfully lower doses of oral levodopa. Key drivers of interest for VY-AADC among these physicians and patients included its one-time treatment, lack of in-dwelling hardware used in deep brain stimulation that often requires replacement and revisions, unique mechanism of action, and magnitude and durability of improvement in patients' motor function. In addition, based on these attributes, neurologists indicated an interest to recommend VY-AADC to patients earlier during the course of Parkinson's disease, before they reached the advanced stage of the disease.

- Plans to begin the global, pivotal Phase 2-3 program for VY-AADC before year-end 2017 and dose the first patient during the second quarter of 2018. Voyager remains on track to file an investigational new drug (IND) application before year-end 2017 with product for VY-AADC manufactured using its baculovirus/Sf9 manufacturing process to support this pivotal program.

- A review of VY-AADC clinical data including the Phase 1b results, historical placebo rates for past gene therapy trials in advanced Parkinson's disease, and a posterior trajectory approach designed to further optimize the intracranial delivery of VY-AADC. Investigators have now dosed a total of six patients in this separate Phase 1 trial designed to further optimize the intracranial delivery of VY-AADC. Administration of VY-AADC with this posterior approach has been well-tolerated in all six patients dosed since the start of the trial and no serious adverse events have been reported. The posterior approach resulted in greater average putaminal coverage (approximately 50%) and reduced average surgical times by two to three hours compared with the transfrontal approach of Cohorts 1 through 3 of the ongoing Phase 1b clinical trial. Voyager continues to expect to enroll additional patients in this trial prior to the start of the pivotal Phase 2-3 program and will continue to follow patients from Cohorts 1 through 3, and those in this posterior trajectory trial, and plans to report updated results from these trials during the first quarter of 2018, prior to the start of patient enrollment in the pivotal program.

- Data in adult non-human primates with novel AAV gene therapy capsids that crossed the blood-brain barrier after a single, intravenous administration resulting in widespread, enhanced gene transfer to the brain and spinal cord. One month after dosing of a transgene encoding both a therapeutic protein (frataxin) and a reporter gene (HA) to facilitate molecular and immunohistochemical measurements, substantial levels of frataxin-HA were expressed in the CNS, including motor neurons throughout the length of the spinal cord, the brain stem, pyramidal neurons in the motor cortex, and neurons in the substantia nigra, and cerebellar dentate nucleus.

- Preclinical data from its Friedreich's ataxia (FA) program in a transgenic mouse model of FA, with a one-time intravenous (IV) dosing of an AAV vector composed of a novel capsid and a frataxin transgene, together with intracerebral dosing of an AAV vector with the same transgene, that led to a rapid halting and reduction of FA disease progression in multiple functional tests of motor behavior. Additional preclinical studies are underway at Voyager including steps to identify a lead clinical candidate for the treatment of FA during 2018.

- An overview of its capsid and delivery optimization efforts resulting in lead candidate selections of VY-SOD101 for the

treatment of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 gene (SOD1) and VY-HTT01 for Huntington's disease. During the third quarter of 2017, Voyager conducted preclinical studies with additional routes of administration in large animal models, including studies with its novel AAV capsids yielding markedly improved bio-distribution and pharmacology with the potential for substantially improved efficacy. Voyager plans further investigation of novel capsids and additional routes of administration for VY-SOD101 and, together with the ongoing efforts on the Huntington's disease and Friedreich's ataxia programs, expects that these efforts will support two IND filings from these programs during 2019.

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. 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.

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 product engine, its ability to develop manufacturing capability for its products, its ability to add new programs to its pipeline, its ability to enter into new partnerships or collaborations, its expected cash, cash equivalents and marketable debt securities at the end of a fiscal period and anticipation for how long expected cash, cash equivalents and marketable debt 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. Such risks and uncertainties include, among others, those related to the initiation and conduct of preclinical studies and clinical trials, the availability of data from clinical trials and the expectations for regulatory submissions and approvals; the continued development of the product engine; Voyager's scientific approach and general development progress; the availability or commercial potential of Voyager's product candidates; the sufficiency of cash resources; and need for additional financing. 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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