Voyager Therapeutics' Novel Capsids Demonstrate Robust Delivery Across the Blood Brain Barrier, Widespread CNS Transduction in Non-Human Primates

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- *Data to be presented at the 24th Annual Meeting of the American Society of Gene and Cell Therapy show up to 1000-fold higher transgene expression in the brain compared with conventional AAV gene therapy following IV administration*

CAMBRIDGE, Mass., May 11, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ: VYGR), a clinical-stage gene therapy company focused on developing life-changing treatments for serious neurological diseases, today will present new preclinical data demonstrating high transduction efficiency of the company's novel adeno-associated virus (AAV) capsids in the central nervous system (CNS) after intravenous dosing in non-human primates. Data will be presented at the 24th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) taking place virtually May 11-14, 2021.

"Efficient targeting of the CNS, including the ability to penetrate the blood brain barrier and transduce target cells, have represented a significant challenge in gene therapy hindering the field's ability to effectively address many serious neurological diseases," Omar Khwaja, M.D., Ph.D., Chief Medical Officer and Head of Research & Development at Voyager. "We've now been able to show that our novel capsid variants not only cross the blood brain barrier but achieve widespread transduction of multiple brain regions including the cortex, thalamus, striatum, cerebellum, brainstem and spinal cord. We believe these capsids can provide a new way forward to treat a broad range of CNS indications, potentially at significantly lower doses than currently available AAV serotypes."

Data will be shared in an oral presentation titled, "RNA-driven Evolution of AAV Capsid Libraries Identifies Variants with High Transduction Efficiency in Non-Human Primate Central Nervous System," (Abstract #51) by Mathieu Nonnenmacher, Ph.D., Director and Head of Capsid Discovery, showing that several capsid variants derived from the company's directed evolution RNA-based screening platform, TRACER ™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA), demonstrated significantly enhanced activity relative to AAV9, the most commonly used vector for CNS gene therapy.

- A subset of capsids showed a 10-fold or higher improvement in transduction of the brain and spinal cord, compared to AAV9.
- The most efficient capsid identified in the work to be presented, TRACER 9P801, displayed more than 1,000-fold higher transgene expression in the brain and 100-fold higher transgene expression in the spinal cord.
- The overall tolerability of 9P801 was favorable and no toxicity was observed in the liver, spinal cord or dorsal root ganglia (DRG).
- Immunohistochemical analysis indicated that 9P801 displayed predominant neuronal tropism and achieved widespread transduction of multiple brain regions including the cortex, thalamus, putamen and brainstem.
- The TRACER platform generates large data sets on engineered capsid performance in a relevant primate species suitable for in silico approaches to optimizing capsid selection for specific cellular targeting and tropism.

Voyager's TRACER system is a broadly-applicable, RNA-based functional screening platform that allows for rapid in vivo evolution of AAV capsids with cell-specific transduction properties in wild-type animals. TRACER candidates were tested individually by low dose intravenous injection and their tropism for the CNS analyzed by measuring transgene RNA expression, viral DNA biodistribution and immunohistochemistry.

Further information about the Company's TRACER platform and pipeline, including novel capsid-enabled new programs, will be shared at an upcoming investor and analyst event in July 2021.

About Voyager Therapeutics

Voyager Therapeutics is a clinical-stage gene therapy company focused on developing life-changing treatments for serious 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. For more information on Voyager Therapeutics, please visit the company's website at www.voyagertherapeutics.com or follow @VoyagerTx on Twitter and 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 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, activities, goals and reporting of results of its research and development programs; Voyager's ability to present preclinical data on its early pipeline progress and novel capsid discovery efforts in non-human primates at scientific meetings and other presentations; Voyager's ability to continue to develop its novel AAV capsids; the ability of Voyager's novel capsids to treat CNS diseases and to treat with lower doses than currently available AAV serotypes, in each instance are forward

looking.

All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes such forward-looking statements 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 severity and length of the COVID-19 health crisis; the initiation and conduct of preclinical studies involving our novel capsid program; the continued development of the Voyager gene therapy platform and Voyager's TRACER system; the applicability of novel capsids identified by the TRACER system to a broad spectrum of CNS and non-CNS disease indications; Voyager's scientific approach and general development progress; the ability to attract and retain highly skilled and experienced scientists and technicians; the ability to create and protect intellectual property related to Voyager's development of novel capsids; and the availability of resources necessary to conduct the novel capsid development program, including access to sufficient number and types of primates suitable for participation in ongoing and planned preclinical research activities.

These statements are also subject to a number of material risks and uncertainties that are described in Voyager's Annual Report on Form 10-K for the year ended December 31, 2020 filed with the Securities and Exchange Commission, as updated by its subsequent filings with the Securities and Exchange Commission. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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