Voyager Therapeutics to Present Broad Set of Translational Data Supporting IV-Delivered, CNS Gene Therapy Programs Advancing Toward Clinical Trials at the ASGCT 27th Annual Meeting

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- Spotlight on new, second-generation, TRACER™-derived AAV capsids; translatability as evidenced by cross-species and receptor data; and activity against therapeutic targets in Alzheimer's disease and ALS -

LEXINGTON, Mass., April 22, 2024 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a biotechnology company dedicated to advancing neurogenetic medicines, today announced that it will present a broad set of data related to its TRACER™ capsid discovery platform and TRACER-driven gene therapy programs across 12 oral and poster presentations at the upcoming American Society of Gene & Cell Therapy's (ASGCT) 27th annual meeting taking place in Baltimore, May 7-11. The findings span the cross-species and receptor-driven translatability, manufacturability, and overall performance of Voyager's novel IV-delivered CNS capsids, which are enabling multiple gene therapies to advance towards clinical trials next year.

"Voyager's novel TRACER-derived capsids underlie 13 partnered programs and three wholly-owned programs to enable IV-delivery of gene therapies for diseases of the central nervous system. Three of those programs now have development candidates selected, and we see the potential for them to enter clinical trials next year," said Todd Carter, Ph.D., Chief Scientific Officer of Voyager Therapeutics. "At ASGCT, we will highlight data on the second-generation capsids that have enabled this progress, as well as preclinical data from our wholly-owned Alzheimer's disease and ALS programs, and multiple studies of cross-species and receptor-based data that increase our confidence in the potential for these capsids to work in humans."

Second-Generation Capsids

- Oral Presentation: Continued directed evolution of VCAP-101 and VCAP-102 identifies second generation capsids with increased brain tropism in non-human primates and mice (#119). Tyler Moyer, Ph.D., Senior Scientist I, Novel Capsid Discovery. Wednesday, May 8, 2024, 4:30 p.m. – 4:45 p.m. ET

Advancements in Wholly-Owned CNS Gene Therapy Programs

- Intravenous administration of BBB-penetrant, MAPT-Silencing, AAV gene therapy provides broad and robust CNS Tau lowering in tauopathy mouse models (#1602). Hechen Bao, Ph.D., Scientist I, and Shiron Lee, M.S., Senior Research Associate I, Neuroscience. Friday, May 10, 2024, 12:00 p.m. ET
- Intravenous delivery of AAV gene therapy for the treatment of SOD1-ALS provides broad SOD1 lowering in NHP (#1647). Michael Grannan, Ph.D., Associate Director, Neuroscience. Friday, May 10, 2024, 12:00 p.m. ET

Mechanism of Action and Cross-Species Translation

- Identification and characterization of a highly conserved cell surface receptor utilized by engineered BBB-penetrant AAV capsids with enhanced brain tropism in non-human primates and mice (#975). Brett Hoffman, Ph.D., Senior Scientist II, Novel Capsid Discovery. Thursday, May 9, 2024, 12:00 p.m. ET
- Establishment of a predictive transcytosis model to recapitulate capsid-receptor interaction and phenotype of BBB-penetrant AAV variants (#976). Ishan Shah, Ph.D., Senior Scientist I, Vector Genome. Thursday, May 9, 2024, 12:00 p.m. ET
- Evaluation of cross-species expression across four species and cellular tropism of VCAP-102, an engineered blood-brain barrier-penetrating AAV derived capsid from TRACER Platform screens (#1452). Hamza Khalid, Research Associate II, Histology. Friday, May 10, 2024, 12:00 p.m. ET
- High-resolution quantitative analysis of multiple AAV capsids in rodent and primate models using multiplexed reporter protein tagging platform (#511). Matthew Child, M.S., Principal Research Associate, Novel Capsid Discovery. Wednesday, May 8, 2024, 12:00 p.m. ET

Reduced Immunogenicity, Developability, and Manufacturing

- Discovery of TRACER AAV capsids escaping pre-existing neutralizing antibodies (#973). Damien Maura, Ph.D., Senior Scientist II, Novel Capsid Discovery. Thursday, May 9, 2024, 12:00 p.m. ET
- Oral Presentation: Developability assessment of novel AAV capsids and payloads at early preclinical stage to enable development of AAV gene therapies (#65). Matteo Placidi, Ph.D., Director, Analytical Sciences. Wednesday, May 8, 2024, 2:45 p.m. – 3:00 p.m. ET
- Machine learning for AAV production-fitness modeling (#974). Daniel Cox, Ph.D., M.S., Senior Scientist, Data Science. Thursday, May 9, 2024, 12:00 p.m. ET
- Comparing CsCl density gradient ultracentrifugation and anion exchange chromatography for the enrichment of full adeno-associated viral (AAV) vectors (#1037). Roberto Facendola, B.S., Scientist II, Downstream Process Development.

Thursday, May 9, 2024, 12:00 p.m. ET

- Development of HEK293 cell line for optimal production of Novel capsids with enhanced brain tropism (#1035). Varshini Venkatesan, M.S., Senior Associate Engineer II, Process Development. Thursday, May 9, 2024, 12:00 p.m. ET

About the TRACER™ Capsid Discovery Platform
Voyager's TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) capsid discovery platform is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier and enhanced central nervous system (CNS) tropism in multiple species, including non-human primates (NHPs). TRACER™ generated capsids have demonstrated superior and widespread gene expression in the CNS compared to conventional AAV capsids as well as cell- and tissue-specific transduction, including to areas of the brain that have been traditionally difficult to reach, while de-targeting the liver and dorsal root ganglia. As part of its external partnership strategy, Voyager has established multiple collaboration agreements providing access to its next-generation TRACER™ capsids to potentially enable its partners' gene therapy programs to treat a variety of diseases.

About Voyager Therapeutics
Voyager Therapeutics, Inc. (Nasdaq: VYGR) is a biotechnology company dedicated to leveraging the power of human genetics to modify the course of – and ultimately cure – neurological diseases. Our pipeline includes programs for Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Parkinson's disease, and multiple other diseases of the central nervous system. Many of our programs are derived from our TRACER™ AAV capsid discovery platform, which we have used to generate novel capsids and identify associated receptors to potentially enable high brain penetration with genetic medicines following intravenous dosing. Some of our programs are wholly owned, and some are advancing with partners including Alexion, AstraZeneca Rare Disease; Novartis Pharma AG; Neurocrine Biosciences, Inc.; and Sangamo Therapeutics, Inc. For more information, 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 "potential" and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager's ability to advance its AAV-based gene therapy programs, including expectations for Voyager's achievement of preclinical and clinical development milestones for its potential development candidates such as the initiation of clinical trials; Voyager's ability to advance gene therapy product candidates under its partnered programs; and the potential for Voyager's novel TRACER capsids to achieve desired results in humans 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 continued development of Voyager's technology platforms, including Voyager's TRACER platform and its antibody screening technology; the ability to initiate and conduct preclinical studies in animal models; the development by third parties of capsid identification platforms that may be competitive to Voyager's TRACER capsid discovery platform; Voyager's ability to create and protect intellectual property rights associated with the TRACER capsid discovery platform, the capsids identified by the platform, and development candidates for Voyager's pipeline programs; the initiation, timing, conduct and outcomes of Voyager's preclinical and clinical studies; the possibility or the timing of Voyager's receipt of program reimbursement, development or commercialization milestones, option exercise, and other payments under Voyager's existing licensing or collaboration agreements; the ability of Voyager to negotiate and complete licensing or collaboration agreements with other parties on terms acceptable to Voyager and the third parties; the ability to attract and retain talented directors, employees, and contractors; and the sufficiency of cash resources to fund its operations and pursue its corporate objectives.

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. 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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