Third Rock Ventures Launches Voyager Therapeutics with $45 Million Series A to Develop Life-Changing Gene Therapies for CNS Disorders

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Company Assembles Leading Clinical and Scientific Founders, Multiple Clinical and Preclinical Product Programs, and Key Strategic Technology Agreements

Cambridge, Mass., February 12, 2014 – Third Rock Ventures, LLC today announced the formation of Voyager Therapeutics, a gene therapy company developing life-changing treatments for fatal and debilitating diseases of the central nervous system (CNS), with a $45 million Series A financing. Voyager's adeno-associated virus (AAV) approach to gene therapy has the potential to transform treatment for a wide range of CNS diseases with one-time therapies that may dramatically improve patients' lives. The company is committed to advancing the field of AAV gene therapy by innovating and investing in areas such as vector optimization and engineering, dosing techniques, as well as process development and production.

"We believe the time is right for gene therapy, and we have assembled the expertise, technology and strategies to convert the promise of AAV gene therapy into meaningful treatments for patients," said Mark Levin, Interim Chief Executive Officer of Voyager and Partner at Third Rock. "Our world-class founders have driven significant advances in their respective fields, and the combination of their deep clinical and scientific knowledge, our experienced management team and Third Rock's investment positions Voyager to deliver breakthrough therapies to patients suffering from devastating CNS disorders."

Voyager was founded by world leaders in the fields of AAV gene therapy, expressed RNA interference (RNAi) and neuroscience:

- Krystof Bankiewicz, M.D., Ph.D., is a translational neurosurgeon and leader in AAV gene therapy who has brought multiple new AAV therapies to the clinic; he is the Kinetics Foundation Chair in Translational Research and a Professor in Residence of Neurological Surgery and Neurology at the University of California, San Francisco (UCSF).

- Guangping Gao, Ph.D., is a leading AAV researcher who has played a key role in the discovery and characterization of new AAV serotypes; he is Director of the University of Massachusetts Medical School (UMMS) Gene Therapy Center & Vector Core, Scientific Director of the UMMS-China Program Office and Professor of Microbiology and Physiology Systems and the Penelope Booth Rockwell Professor in Biomedical Research at UMMS.

- Mark Kay, M.D., Ph.D., is a leading researcher in the fields of gene therapy and AAV biology, including the identification of new AAV capsids; he is the Head of the Division of Human Gene Therapy and Professor in the Departments of Pediatrics and Genetics at Stanford University School of Medicine; he is one of the founders of the American Society of Gene and Cell Therapy and is currently the Editor of Human Gene Therapy.

- Phillip Zamore, Ph.D., is a leader and innovator in the understanding of RNAi and development of related therapeutic approaches, including expressed RNAi; he is a Howard Hughes Medical Institute Investigator, the Gretchen Stone Cook Chair of Biomedical Sciences at UMMS, Professor of Biochemistry and Molecular Pharmacology and Co-Director of the RNA Therapeutics Institute at UMMS.

Voyager is launching with multiple clinical and preclinical product programs for CNS diseases in dire need of effective new therapies, including a Parkinson's disease program in an ongoing Phase 1b study with our collaborators at UCSF, as well as preclinical programs for a monogenic form of amyotrophic lateral sclerosis (ALS) and Friedreich's ataxia. Depending on the disease, the company's gene therapy products will use either gene replacement or gene knockdown techniques. By significantly increasing or decreasing production of relevant proteins at targeted sites within the CNS, the goal is to address the underlying biology of the disease and make a meaningful difference for patients.

"CNS disorders are one of the largest areas of unmet medical need," said Philip Reilly, M.D., J.D., Interim Chief Medical Officer of Voyager and Venture Partner at Third Rock. "By expressing the right amount of protein in the right cell at the right time or knocking down a gene to decrease levels of a disease-causing protein, we believe our gene therapy approach is uniquely suited to targeting these devastating diseases and has the potential to fundamentally change the face of treatment for many patients."

Voyager has entered into license and other agreements with UMMS, UCSF and Stanford University to access relevant technology and data, including certain rights to intellectual property related to expressed RNAi technology discovered at UMMS, clinical data related to the Parkinson's disease program from UCSF, and certain rights to AAV intellectual property developed at Stanford. In order to accelerate its research and development efforts, Voyager has also entered into a broad strategic collaboration with UMMS, a leader in AAV research and translation.

Over the past 10 years, AAV has emerged as a highly promising and attractive approach to gene therapy. In clinical trials, AAV has been shown to be a safe and effective gene therapy delivery vehicle. Advances in AAV vector development and related dosing techniques that enable widespread gene delivery in the brain and spinal cord have made AAV particularly well-suited for the treatment of CNS diseases. In addition, improvements in related production technology and approaches have made AAV production more easily scalable and cost effective to meet commercial requirements.

Voyager's founding management team has deep expertise and a track record of building exceptional life science companies. In addition to Mr. Levin, who has more than 30 years of experience building and operating leading biotech companies, and Dr. Reilly, a highly respected clinical geneticist who leads Third Rock's efforts in rare diseases, the team includes: Steven Paul, M.D., Interim President of R&D and Venture Partner at Third Rock, who spent 17 years at Eli Lilly in leadership roles of increasing responsibility, including Vice President of Discovery Research and Neuroscience Research, and President of the Lilly Research Laboratories, Dr. Paul also served as Scientific Director of the National Institute of Mental Health having served as a Branch Chief at the National Institutes of Health (NIH) for 10 years prior to joining Eli Lilly; Jim Geraghty, Interim Chief Business Officer and Entrepreneur-in-Residence at Third Rock, who spent 20 years at Genzyme in leadership roles across a variety of functions, including as a Senior Vice President and Officer; Dinah Sah, Ph.D., Interim Senior Vice President of Neuroscience and former Vice President of Research at Alnylam; Jeff Goater, Vice President of Business Development and former Managing Director at Evercore Partners; Rob Kotin, Ph.D., Vice President of Production and former Senior Investigator at the NIH, where he was the co-inventor of the baculovirus-based AAV production system; and James McLaughlin, Head of Operations and former Associate at Third Rock.

A core part of our strategy is to work closely with and learn from patient advocacy groups, and we have developed strong relationships with leading patient education and research foundations such as the Friedreich's Ataxia Research Alliance (FARA) and The Michael J. Fox Foundation for Parkinson's Research, which partly funds Dr. Bankiewicz's work with AAV gene therapy.

About Voyager Therapeutics

Voyager Therapeutics is a gene therapy company developing life-changing treatments for fatal and debilitating diseases of the central nervous system (CNS). Voyager is committed to advancing the field of AAV (adeno-associated virus) gene therapy through innovation and investment in vector optimization and engineering, dosing techniques, as well as process development and production. The company's initial pipeline is focused on CNS diseases in dire need of effective new therapies, including Parkinson's disease, a monogenic form of amyotrophic lateral sclerosis (ALS), and Friedreich's ataxia. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics was launched in 2014 with funding from leading life sciences investor Third Rock Ventures and is headquartered in Cambridge, Mass. For more information, please visit www.voyagertherapeutics.com.

About Third Rock Ventures

Third Rock Ventures is a leading healthcare venture firm focused on investing and launching companies that make a difference in people's lives. The Third Rock team has a unique vision for ideating and building transformative healthcare

companies. Working closely with our strategic partners and entrepreneurs, Third Rock has an extensive track record for managing the value creation path to deliver exceptional performance. For more information, please visit the firm's website at www.thirdrockventures.com

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