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Passage Bio Launches with \$115.5 Million Series A to Develop AAV-Delivered Therapeutics to Treat Rare Monogenic CNS Diseases

Industry veterans Stephen Squinto, Ph.D. and Tachi Yamada, M.D. team up with gene therapy pioneer James Wilson, M.D., Ph.D. to launch new world-class CNS-focused gene therapy company

PHILADELPHIA, Feb. 14, 2019 (GLOBE NEWSWIRE) -- Passage Bio today debuted with a \$115.5 million Series A financing led by OrbiMed Advisors and joined by Frazier Healthcare Partners, Versant Ventures, New Leaf Venture Partners, Vivo Capital and Lilly Asia Ventures. The funding will be used to develop a portfolio of five therapeutic candidates to treat rare monogenic CNS diseases. These programs are being developed under a research, collaboration and license agreement with the University of Pennsylvania (Penn) and its Gene Therapy Program (GTP) together with the Penn Orphan Disease Center (ODC), with the support of co-founder James M. Wilson, M.D., Ph.D. Passage Bio also has an option to fund the preclinical development of up to seven additional rare monogenic CNS indication programs at the GTP and license new intellectual property arising from these programs from Penn.

Co-founder and interim Chief Executive Officer at Passage Bio, Stephen Squinto, Ph.D., is a Venture Partner at OrbiMed Advisors with more than 25 years of biotechnology industry experience. Dr. Squinto was a co-founder of Alexion Pharmaceuticals Inc. and recently served as its Executive Vice President and Chief Global Operations Officer. While at Alexion, Dr. Squinto was involved in the discovery, development and commercialization of Soliris, one of the world's most successful orphan drug products for patients with paroxysmal nocturnal hemoglobinuria, atypical hemolytic uremic syndrome and generalized myasthenia gravis.

"Passage Bio's development portfolio presents an unparalleled opportunity to transform the lives of patients with rare monogenic CNS diseases," said Dr. Squinto. "We look forward to continuing progress in this exciting field of therapeutics and advancing our lead programs in GM1 gangliosidosis and frontotemporal dementia into the clinic in early 2020."

Co-founder and Chairman of the Board at Passage Bio, Tachi Yamada, M.D. is a Venture Partner at Frazier. Prior to joining Frazier he was Chief Medical and Scientific Officer at Takeda Pharmaceuticals and has served as Chairman of Research and Development at GlaxoSmithKline.

"We co-founded Passage Bio with a shared commitment to serving patients with high unmet needs. Both Dr. Wilson and I have been working in the gene therapy field for over 30 years, and this deep understanding will be instrumental as we grow and advance this new world-class gene therapy company," said Dr. Yamada. "This financing will allow us to continue to build out our industry-leading team and advance our therapies into the clinic as rapidly as possible."

Co-founder and Chief Scientific Advisor at Passage Bio, James M. Wilson, M.D., Ph.D., is Director, Gene Therapy Program; Rose H. Weiss Professor and Director, Orphan Disease Center; Professor of Medicine and Pediatrics, Department of Medicine at the Perelman School of Medicine at the University of Pennsylvania. Dr. Wilson's laboratory has made seminal contributions to the technology of gene transfer and has paved the way for translation of these technologies into the clinic. He has published over 550

papers and is named on over 110 patents. After launching his faculty career in the Howard Hughes Medical Institute at the University of Michigan, he moved to the University of Pennsylvania in 1993. Dr. Wilson completed his training in Internal Medicine at the Massachusetts General Hospital followed by a postdoctoral fellowship at the Whitehead Institute, where he began his work in gene therapy.

“We believe this is a truly unique partnership, which gives Passage access to certain Penn AAV technologies developed at the GTP, our strong preclinical translational science capabilities and orphan drug development know-how,” said Dr. Wilson. “Our team at Penn is extremely experienced and has been on the cutting edge of AAV research for over 20 years. We are confident in this team’s ability to move new treatments for rare CNS monogenic diseases through clinical development in an effort to one day provide new treatment options for patients with chronic unmet needs with high mortality.”

Additional members of Passage Bio’s Board of Directors include Carl Gordon, managing partner of OrbiMed Advisors; Patrick Heron managing general partner at Frazier Healthcare Partners and Tom Woiwode, Ph.D., managing director at Versant Ventures.

Initial Indications: GM1 and FTD

GM1 gangliosidosis (GM1) is an autosomal recessive genetic disorder, caused by an inactivating mutation of the lysosomal enzyme β -galactosidase (GLB1), which is required for the degradation of GM1 ganglioside and keratan sulfate. The infantile type of GM1 is the most common and severe form of the disease, typically with gait abnormalities by four months of age and developmental regression by six months. Many infantile GM1 patients experience rapid regression with mortality by approximately two years of age. There are no disease modifying therapies for GM1.

Frontotemporal dementia (FTD) typically presents in the sixth or seventh decade with progressive impairment of executive function, language and social interaction. These symptoms are associated with a characteristic pattern of neurodegeneration affecting the frontal and temporal cortices. In 5-10% of FTD patients, pathogenic loss-of-function mutations can be identified in the gene encoding progranulin, a ubiquitous lysosomal protein. Patients universally exhibit a progressive course, with an average survival of eight years from symptom onset. There are currently no disease modifying therapies for FTD.

About Passage Bio

Passage Bio is a fully integrated gene therapy company with a mission to develop a portfolio of five life-transforming AAV-delivered therapeutics for the treatment of rare monogenic central nervous system diseases. The company is based in Philadelphia, PA and has a research, collaboration and license agreement with the University of Pennsylvania and its Gene Therapy Program (GTP) as well as the Orphan Disease Center (ODC). Pursuant to the research collaboration, GTP conducts IND-enabling preclinical work, and Passage Bio is responsible for clinical development, regulatory, manufacturing and commercialization of all product candidates. The ODC is responsible for natural history studies, KOL engagement, and patient advocacy outreach. The company recently completed a \$115.5 million Series A financing with investments from OrbiMed, Frazier Healthcare Partners, Versant Ventures, New Leaf Venture Partners, Vivo Capital and Lilly Asia Ventures.

Penn’s Financial Disclaimer

Dr. Wilson, the Wilson Family Trust, Julie Johnston, and Monique Molloy, all from Penn, hold founder shares in Passage Bio. Penn and GTP will receive sponsored research funding from Passage Bio, and as an inventor of the licensed technology Dr. Wilson and Penn may receive additional financial benefits under the license in the future. Penn also holds an equity interest in Passage Bio, and Dr. Wilson is compensated for his scientific advisory position.

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