Passage Bio Announces Launch of Natural History Study to Evaluate Patients with GM1 Gangliosidosis

August 13, 2019

PHILADELPHIA, Aug. 13, 2019 (GLOBE NEWSWIRE) -- Passage Bio, a genetic medicines company developing AAV-delivered gene therapies for the treatment of rare monogenic central nervous system diseases, today announced the launch of a Natural History Study to evaluate patients with Type 1 and Type 2 GM1 gangliosidosis, a rare autosomal recessive genetic disorder that progressively destroys nerve cells in the brain and spinal cord. The study is being conducted by the Orphan Disease Center (ODC) in the Perelman School of Medicine at the University of Pennsylvania.

“There is a limited understanding of GM1 gangliosidosis, particularly in pediatric patients, and thus it’s important that we sponsor this Natural History Study in order to gather information on the symptoms and progression of this incredibly devastating rare disease,” said Stephen Squinto, co-founder and interim chief executive officer at Passage Bio. “Data from this foundational study will be used to help rationally design our clinical development programs with the goal of fully addressing the unique and clinically meaningful challenges of GM1.”

The ODC will initiate this study at several sites in the US, Europe and Brazil to collect information on the disease’s natural course and outcomes, without treatment or intervention, and to identify demographic, genetic, environmental and other factors that may be associated with this disease. The study will look at baseline characteristics and key clinical assessments out to three years, such as survival rates, feeding tube placement, scores on several validated clinical assessments and all planned first-in-human exploratory endpoints, as well as parent-reported outcomes, including seizure onset and frequency and the Pediatric Quality of Life (PedsQL) assessment.

The findings from this study are expected to be reported at a scientific meeting and will inform a clinical development program expected to be initiated in the first half of 2020, using a new experimental gene therapy being developed by Passage Bio.

About GM1
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.

About Passage Bio
Passage Bio is a privately-held fully integrated genetic medicines company with a mission to develop a portfolio of 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. In early 2019, the company 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.

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Source: Passage Bio