Passage Bio Announces Third Gene Therapy Development Program in Krabbe Disease and Supports Million Dreams Fundraising Gala

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PHILADELPHIA, May 30, 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 that its third clinical trial program will be for infantile Krabbe disease, an inherited disease that causes progressive damage to the nervous system. The company also announced its support of the Million Dreams Fundraising Gala being held tomorrow, May 31st in Minneapolis, MN.

“Passage Bio is committed to serving patients with life threatening, devastating diseases, and Krabbe is certainly no exception,” said Stephen Squinto, co-founder and interim chief executive officer at Passage Bio. “With our deep orphan drug development know-how, we have selected what we believe to be an optimal proprietary capsid and delivery route and look forward to advancing this program into the clinic next year. Additionally, we are honored to partake in the Million Dreams Fundraising Gala, as well as being actively involved in the greater Krabbe community by engaging with patients in a meaningful way and raising awareness for this debilitating illness.”

“Krabbe disease is one of the most challenging neurodegenerative lysosomal diseases, and the preclinical data for this program are highly encouraging,” said Tim Cox, M.D., Professor of Medicine Emeritus and Director of Research at the University of Cambridge, who specializes in the treatment of lysosomal diseases by gene therapy. “I greatly look forward to seeing the outcome of this work and to enrollment of patients in this much-needed study.”

The company plans to initiate a clinical trial in Krabbe disease in 2020. The trial will be a global, open-label, multi-center, dose escalation study of GTP-206 to evaluate the safety, tolerability, and exploratory efficacy endpoints in pediatric subjects with the infantile form of Krabbe disease. GTP-206 is being developed under a research, collaboration and license agreement with the University of Pennsylvania and its Gene Therapy Program.

“Our hope is that through the ground-breaking work of Passage Bio and other companies in the gene therapy field, there will come a day in which each patient receives an early diagnosis, followed by state-of-the-art care and lives a life free of disease,” said Stacy Pike-Langenfeld, co-founder and president of KrabbeConnect. “I would like to thank the Passage team for their sponsorship of the Million Dreams Fundraising Gala and look forward to future opportunities for our organizations to support each other in our shared commitment to helping patients and their families who are impacted by this devastating disease.”

About Krabbe Disease
Krabbe disease is an autosomal recessive lysosomal storage disease, caused by mutations in the gene encoding the hydrolytic enzyme galactosylceramidase (GALC), which results in the destruction of the myelin of nerve cells in the brain and throughout the nervous system. The infantile type of Krabbe disease is the most common and severe form, typically manifesting before six months of age, with rapid progression and median survival of approximately two years in the majority of these patients. The estimated incidence of early and late-stage infantile Krabbe disease is one in 100,000 live births.

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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