Passage Bio Announces New Gene Therapy Development Program for Patients with Charcot-Marie-Tooth Neuropathy Type 2A

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PHILADELPHIA, Sept. 09, 2019 (GLOBE NEWSWIRE) -- Passage Bio, a genetic medicines company developing AAV-delivered gene therapies for the treatment of rare monogenic central nervous system (CNS) diseases, today announced that it has licensed a sixth gene therapy development program under its research, collaboration and license agreement with the University of Pennsylvania and its Gene Therapy Program (GTP). The license is for the clinical development of a potential treatment for patients with Charcot-Marie-Tooth Neuropathy Type 2A (CMT2A).

“CMT2A affects almost all of the severe dominant CMT2 cases and patients suffering from this rare disease experience progressive muscle atrophy of legs and arms, with no FDA-approved curative or symptomatic medications available,” said Dr. Stephen Squinto, co-founder and interim chief executive officer at Passage Bio. “The Gene Therapy Program at Penn has developed AAV vectors and delivery methods to target the nerve cells that are affected in CMT2A, raising the possibility of slowing or preventing progression of the disease by tackling the underlying genetic cause. Passage Bio will develop this experimental therapy, designed to restore the normal function of the MFN2 gene, which is mutated in patients with CMT2A, and we look forward to initiating a clinical trial in the near future.”

“Just one year after we formally launched our gene therapy program, we are witnessing two major players in the field working collaboratively to develop potential treatments for one of the more common types of CMT,” said Gilles Bouchard, Chairman of the Charcot-Marie-Tooth Association. “We are delighted to partner with Passage Bio and Penn in this effort and to contribute key elements of the Strategy to Accelerate Research (STAR) program, such as pre-clinical and clinical assets, access to top CMT experts and engaging the CMT community.”

The clinical trial is anticipated to be a global, open-label, multi-center, dose escalation study to evaluate the safety, tolerability and exploratory efficacy endpoints in subjects with CMT2A.

Charcot-Marie-Tooth Neuropathy Type 2A
Charcot-Marie-Tooth disease encompasses a heterogeneous group of inherited, progressive, chronic peripheral neuropathies. While many types of CMT affect the myelin sheath of nerves, CMT2 results from the degeneration of the axons of neurons. CMT2A, caused by dominantly inherited mutations in the MFN2 gene, is the most common type of CMT2. Patients with CMT2A usually begin to experience progressive weakness in childhood and most become wheelchair dependent.

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 at Penn. The GTP conducts the IND-enabling preclinical work and Passage Bio conducts all clinical development, regulatory strategy and commercialization activities. The company has a development portfolio of six product candidates, with the option to license six more, with lead programs in GM1 gangliosidosis, frontotemporal dementia (FTD) and Krabbe disease, all of which are planned to be in the clinic in 2020. Since launching the company, Passage Bio has raised $225.5 million with investments from OrbiMed, Versant Ventures, Frazier Healthcare Partners, Access Biotechnology, Lily Asia Ventures, New Leaf Venture Partners, Vivo Capital, and Boxer Capital of Tavistock Group, among others.

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