Passage Bio Receives Rare Pediatric Disease Designation for PBGM01 for Patients with GM1 Gangliosidosis

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– PBGM01 to enter the clinic in fourth quarter of 2020 –

PHILADELPHIA, May 21, 2020 (GLOBE NEWSWIRE) -- Passage Bio, Inc. (NASDAQ: PASG), a genetic medicines company focused on developing transformative therapies for rare, monogenic central nervous system disorders, today announced the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to PBGM01 broadly for the treatment of GM1 gangliosidosis. PBGM01 is an AAV-delivery gene therapy currently being developed for the treatment of infantile GM1 gangliosidosis (GM1) and has previously been granted Orphan Drug designation.

"This is the second regulatory designation we have received from the FDA for our lead program in GM1 and reflects the high unmet need in this patient population," said Bruce Goldsmith, Ph.D, president and chief executive officer of Passage Bio. "As a company we are committed to developing therapies that transform the lives of patients suffering from serious life-threatening CNS disorders. We believe that PBGM01 has the potential to restore developmental progression, enabling patients to achieve additional milestones and improve quality of life. We look forward to advancing PBGM01 into clinical testing later this year."

RPD designation is granted by the FDA to encourage treatments for serious or life-threatening diseases primarily affecting children 18 years of age and younger and fewer than 200,000 people in the United States. Under the RPD program, a sponsor who receives approval for a drug or biologic for a "rare pediatric disease" may qualify for a priority review voucher that may be sold or transferred.

Passage anticipates starting a Phase 1/2 trial for PBGM01 for the treatment infantile GM1 in the fourth quarter of 2020. The trial will be an open-label, dose escalation study of PBGM01 administered by a single injection into the intra cisterna magna, or ICM, in pediatric subjects with infantile GM1.

About GM1
GM1 gangliosidosis (GM1) is a rare and often life-threatening monogenic recessive lysosomal storage disease caused by mutations in the GLB1 gene, which encodes lysosomal acid beta-galactosidase (β-gal). Reduced β-gal activity results in the accumulation of toxic levels of GM1 ganglioside in neurons throughout the brain, causing rapidly progressing neurodegeneration. GM1 manifests as a continuum of disease and is most severe in the Infantile form, which is characterized by onset in the first 6 months of life with hypotonia (reduced muscle tone), progressive CNS dysfunction, and rapid developmental regression. Life expectancy for infants with GM1 is two to four years, and infantile GM1 represents approximately 62.5% of the incidence of 0.5 to 1 in 100,000 live births. Currently, there are no approved disease-modifying therapies available.

About PBGM01
PBGM01 is an AAV-delivery gene therapy currently being developed for the treatment of infantile GM1, in which patients have mutations in the GLB1 gene causing little or no residual β-gal enzyme activity and subsequent neurodegeneration. PBGM01 utilizes a next-generation AAVhu68 capsid administered through intra-cisterna magna (ICM) to deliver a functional GLB1 gene encoding β-gal to the brain and peripheral tissues. By reducing the accumulation of GM1 gangliosides, PBGM01 has the potential to reverse neuronal toxicity, thereby restoring developmental potential. In preclinical models, PBGM01 has demonstrated broad brain distribution and wide uptake of the β-gal enzyme in both the central nervous system (CNS) and critical peripheral organs, suggesting potential treatment for both the CNS and peripheral manifestations of GM1. The Company has received Orphan Drug and Rare Pediatric Disease designation for PBGM01 for patients with GM1 and plans to initiate a Phase 1/2 trial in the fourth quarter of 2020 with initial 30 day safety and biomarker data expected in the late first half of 2021.

About Passage Bio
Passage Bio is a genetic medicines company focused on developing transformative therapies for rare, monogenic central nervous system disorders with limited or no approved treatment options. 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). The GTP conducts discovery and IND-enabling preclinical work and Passage Bio conducts all clinical development, regulatory strategy and commercialization activities under the agreement. The company has a development portfolio of six product candidates, with the option to license eleven more, with lead programs in GM1 gangliosidosis, frontotemporal dementia and Krabbe disease.

Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of, and made pursuant to the safe harbor provisions of, the Private Securities Litigation Reform Act of 1995, including, but not limited to: our expectations about timing and execution of anticipated milestones, including our planned IND submissions, initiation of clinical trials and the availability of clinical data from such trials; our expectations about our collaborators' and partners' ability to execute key initiatives; the ability to receive and use an RPD voucher; and the ability of our lead product candidates to treat the underlying causes of their respective target monogenic CNS disorders. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including: our ability to develop, obtain regulatory approval for and commercialize our product candidates; the timing and results of preclinical studies and clinical trials; the risk that positive results in a preclinical study or clinical trial may not be replicated in subsequent trials or success in early stage clinical trials may not be predictive of results in later stage clinical trials; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; the occurrence of adverse safety events; failure to protect and enforce our intellectual property, and other proprietary rights; failure to

successfully execute or realize the anticipated benefits of our strategic and growth initiatives; risks relating to technology failures or breaches; our dependence on collaborators and other third parties for the development of product candidates and other aspects of our business, which are outside of our full control; risks associated with current and potential delays, work stoppages, or supply chain disruptions caused by the coronavirus pandemic; risks associated with current and potential future healthcare reforms; risks relating to attracting and retaining key personnel; failure to comply with legal and regulatory requirements; risks relating to access to capital and credit markets; and the other risks and uncertainties that are described in the Risk Factors section in documents the company files from time to time with the Securities and Exchange Commission (SEC), and other reports as filed with the SEC. Passage Bio undertakes no obligation to publicly update any forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.

For further information, please contact:

Investors:
Sarah McCabe
Stern Investor Relations, Inc.
212-362-1200
sarah.mccabe@sternir.com

Media:
Azeem Zeekrya
HDMZ
312-506-5244
azeem.zeekrya@hdmz.com