



## Passage Bio's PBGM01 Receives Orphan Drug Designation from EMA for Treatment of GM1 Gangliosidosis

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PHILADELPHIA, Oct. 26, 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 that the European Commission (EC) has granted orphan drug designation for the company's lead gene therapy candidate PBGM01 for the treatment of GM1 gangliosidosis (GM1). PBGM01, an adeno-associated virus (AAV)-delivery gene therapy, has previously been granted Orphan Drug Designation and Rare Pediatric Disease Designation by the U.S. Food and Drug Administration (FDA) for the treatment of GM1. GM1 is a rare and often life-threatening central nervous system disorder with no approved disease-modifying therapies available.

"GM1, most common and severe in infants, results in rapid neurodegeneration and is simply devastating for patients and their families," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "PBGM01 is a potentially transformative gene therapy that may lead to the preservation of neurological function and an improvement in developmental potential and survival in patients with GM1. Receiving orphan drug designation for PBGM01 in the European Union is an important milestone that will propel our efforts to bring this much-needed therapy to waiting patients."

The EC grants orphan drug designation based on a positive opinion issued by the EMA Committee for Orphan Medicinal Products (COMP). To qualify for orphan designation, an investigational medicinal product must be intended to treat a chronically debilitating or life-threatening condition that affects fewer than five in 10,000 people across the European Union and there is no effective treatment approved in EU. With EMA orphan drug designation, Passage Bio will receive certain benefits and incentives including 10-year market exclusivity for the approved therapeutic indication once PBGM01 receives marketing authorization as well as clinical protocol assistance and reduced regulatory fees.

Passage Bio expects to initiate dosing of PBGM01 in a Phase 1/2 trial late in the fourth quarter of 2020 or early in the first quarter of 2021, with initial 30-day safety and biomarker data expected in the late first half of 2021. 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 ( $\beta$ -gal). Reduced  $\beta$ -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.

### 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  $\beta$ -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  $\beta$ -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  $\beta$ -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.

### About Passage Bio

At Passage Bio (Nasdaq: PASG), we are on a mission to provide life-transforming gene therapies for patients with rare, monogenic CNS diseases that replace their suffering with boundless possibility, all while building lasting relationships with the communities we serve. Based in Philadelphia, PA, our company has established a strategic collaboration and licensing agreement with the renowned University of Pennsylvania's Gene Therapy Program to conduct our discovery and IND-enabling preclinical work. This provides our team with unparalleled access to a broad portfolio of gene therapy candidates and future gene therapy innovations that we then pair with our deep clinical, regulatory, manufacturing and commercial expertise to rapidly advance our robust pipeline of optimized gene therapies into clinical testing. As we work with speed and tenacity, we are always mindful of patients who may be able to benefit from our therapies. More information is available at [www.passagebio.com](http://www.passagebio.com).

### 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; our expectations about manufacturing plans and strategies; our expectations about cash runway; 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 and obtain regulatory

approval for our product candidates; the timing and results of preclinical studies and 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; 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; failure to protect and enforce our intellectual property, and other proprietary rights; our dependence on collaborators and other third parties for the development and manufacture 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; 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 and Zofia Mita  
Stern Investor Relations, Inc.  
212-362-1200  
[sarah.mccabe@sternir.com](mailto:sarah.mccabe@sternir.com)  
[zofia.mita@sternir.com](mailto:zofia.mita@sternir.com)

**Media:**

Gwen Fisher  
Passage Bio  
215-407-1548  
[gfisher@passagebio.com](mailto:gfisher@passagebio.com)