Passage Bio Announces Pre-Clinical Data Presentation at Annual ASGCT Meeting

May 11, 2021

- *Passage Bio collaborator University of Pennsylvania's Gene Therapy Program to present poster regarding development of a novel mouse model for evaluating efficacy of adeno-associated virus gene therapy for metachromatic leukodystrophy (MLD)*
- *Passage Bio gene therapy candidate, PBML04, showed preliminary signs of efficacy in novel mouse model of MLD*

PHILADELPHIA, May 11, 2021 (GLOBE NEWSWIRE) -- Passage Bio, Inc. (Nasdaq: PASG), a clinical-stage genetic medicines company focused on developing transformative therapies for rare monogenic central nervous system (CNS) disorders, announced today the presentation by its collaborator University of Pennsylvania's Gene Therapy Program (GTP) of a digital poster at the 24th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT). Gourav Roy Choudhury, Ph.D., a senior research investigator at GTP, will report on the development and utility of a novel Arylsulfatase A-deficient mouse model in reproducing key aspects of human metachromatic leukodystrophy (MLD) neuropathology to enable evaluation of efficacy of adeno-associated virus (AAV) gene therapy. Preliminary findings showed the administration of Passage Bio's gene therapy candidate PBML04 (AAVhu68.GTP-207) significantly reduced the neurological deficits of MLD in this model.

"This novel animal model makes it possible to evaluate the pre-clinical efficacy of our AAV-mediated gene therapy for MLD," said Bruce Goldsmith, Ph.D., President and Chief Executive Officer of Passage Bio. "One of the major challenges in developing treatments for MLD has been the limited availability of mouse models and scarcity of phenotype in existing models. This model reproduces important aspects of human leukodystrophy neuropathology, including key biomarkers. We are encouraged by the preliminary signs of efficacy we saw with PBML04 in this study."

The biomarkers used in the Arylsulfatase A-deficient mouse model were LAMP1, GFAP, and several sulfatide species (C16:0 and C18:0). Passage Bio's gene therapy candidate PBML04, which is at the IND-enabling stage of development, utilizes an AAV viral vector to deliver a codon-optimized gene sequence encoding functional ARSA enzyme.

ASGCT Presentation Details
Title: Development and Characterization of a Novel Arylsulfatase A-deficient Mouse Model of Metachromatic Leukodystrophy to Evaluate the Efficacy of Gene Therapy
Date and time: Tuesday, May 11, 2021, 8:00am – 10:00am ET
Presenter: Gourav Roy Choudhury, Ph.D., University of Pennsylvania
Abstract number: 494

About MLD
MLD is a monogenic autosomal recessive sphingolipid storage disease caused by mutations in the gene encoding the lysosomal enzyme ARSA. Patients with MLD display progressive leukodystrophy (demyelination) in the central and peripheral nervous systems, neuronal cell death, and subsequent loss of all motor and cognitive function, resulting in premature death, especially in patients with early disease onset.

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 enhanced 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.

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 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 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.

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