Passage Bio Announces Plan to Deliver on Multiple Meaningful Catalysts in 2021

January 11, 2021

– Investigational New Drug Applications (IND) submitted for PBFT02 for Frontotemporal Dementia with Granulin Mutation (FTD-GRN) and PBKR03 for Krabbe Disease –

– Three clinical programs to begin in 1H21 –

– Metachromatic leukodystrophy program, PBML04, advanced to IND-enabling phase –

– Additional option exercised from Penn's world-class Gene Therapy Program for undisclosed program focused on an adult CNS disorder –

– New manufacturing laboratory to support analytical capabilities, clinical product testing and assay validation slated to open in 2Q21 –

– PBFT02 for FTD-GRN becomes latest of company's three most advanced programs to receive FDA orphan drug designation –

PHILADELPHIA, Jan. 11, 2021 (GLOBE NEWSWIRE) -- Passage Bio, Inc. (Nasdaq: PASG), a genetic medicines company focused on developing transformative therapies for rare, monogenic central nervous system (CNS) disorders, today provided a corporate outlook detailing the expansion of the company's pipeline, new regulatory filings, projected program milestones, and research and development progress. Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio, will highlight the company's recent progress and plans for the future in his presentation today at the 39th Annual J.P. Morgan Healthcare Conference.

In his remarks, Dr. Goldsmith will highlight several components of the Company's progress and timelines, including:

- Planned initiation of patient dosing in clinical studies for three novel adeno-associated virus (AAV) gene therapies:
  - Received FDA and MHRA clearance for PBGM01 for infantile GM1 gangliosidosis (GM1)
  - Submitted IND for PBKR03 for the treatment of Krabbe disease
  - Submitted IND for PBFT02 for treatment of Frontotemporal Dementia (FTD) with granulin (GRN) mutation.
- Advancement of PBML04 for the treatment of metachromatic leukodystrophy (MLD) into IND-enabling studies
- License of a seventh program from the University of Pennsylvania (Penn) for the treatment of an adult rare monogenic CNS disorder
- Planned opening of a manufacturing laboratory to support analytical capabilities, clinical product testing and assay validation

"It is widely reported that there are approximately 7,000 rare diseases. Less discussed is that out of these 7,000 rare diseases, there are more than 790 rare monogenic CNS diseases, the area where we are focused," said Dr. Goldsmith. "The diseases we are targeting are particularly devastating and have no approved disease-modifying treatment options, which means there is tremendous opportunity to make a difference for patients. At Passage Bio, we believe we are well positioned with our collaboration with Penn's Gene Therapy Program, our corporate model, robust pipeline, manufacturing capability, strong cash position, and experienced team to succeed in our mission to deliver on the promise of gene therapies for patients with rare monogenic CNS disorders."

Passage Bio's collaboration with Penn's world-class Gene Therapy Program (GTP) provides the company with access to cutting-edge research, expertise and next-generation technologies. GTP is recognized worldwide for its innovative gene therapy capabilities, AAV vector technology manufacturing, and IND expertise. Through the Penn GTP collaboration, Passage Bio has licensing options for a total of 17 gene therapy research programs focused on rare monogenic disorders of the CNS through 2025. The company has exercised seven options to date, including four programs for pediatric and three programs for adult rare monogenic CNS disorders.

"Through our relationship with GTP coupled with our business model, we have a well-defined path for clinical success," explained Dr. Goldsmith. "We believe our pipeline has a higher probability of technical and regulatory success, in part because we are able to optimize delivery approaches, select candidates based on empirical research findings and integrate next-generation innovations from GTP as appropriate. Additionally, all our current therapies are designed for direct delivery to the CNS, which we believe offers a number of benefits, including avoiding crossing the blood brain barrier, better CNS biodistribution and lower dosing compared to systemic delivery."

Dr. Goldsmith added: "We spent 2020 establishing a solid foundation for our company to execute seamlessly against our ambitious future plans. Our progress has laid the groundwork for clinical trial preparedness and manufacturing readiness, which will be critical to successfully deliver on several meaningful catalysts this year."

The company's three most advanced programs are: PBGM01 for infantile GM1 and PBKR03 for Krabbe disease, which are pediatric programs; and PBFT02 for FTD-GRN, which is an adult program. Passage Bio is also continuing to progress preclinical pediatric programs PBCM06 for Charcot-Marie-Tooth neuropathy type 2A (CMT2A) and PBML04 for MLD, which was recently advanced into IND-enabling studies. The company's additional adult preclinical programs are PBAL05 for amyotrophic lateral sclerosis (ALS) and a seventh program, for which the company recently exercised an option and has not yet disclosed the therapeutic target.

Significant Pipeline and Corporate Advancement

The company's lead program PBGM01 is entering the clinic to study its safety and efficacy in addressing infantile GM1, a rare and often life-threatening CNS disorder with no approved disease-modifying therapies. The global Phase 1/2 study, Imagine-1, is expected to enroll the first patient in the first quarter of 2021 and is expected to report initial 30-day safety and biomarker data mid-year 2021. PBGM01 has received regulatory agency clearance from FDA and MHRA. The investigational therapy has also received orphan drug and rare pediatric disease designations from FDA, as well as orphan drug designation from the European Medicines Agency for the treatment of GM1.

The company has submitted investigational new drug (IND) applications to FDA for Phase 1/2 clinical studies of PBFT02 in FTD-GRN, a devastating form of early onset dementia; and PBKR03 in Krabbe disease, a pediatric disease with rapid progression, typically resulting in death by 2 years old. The company recently received orphan drug designation from FDA for PBFT02 for FTD-GRN. PBKRO3 has orphan drug and rare pediatric disease designations from FDA for treatment of Krabbe disease. Passage Bio anticipates the start of both clinical programs in the first half of 2021. Initial data from these trials are anticipated to potentially readout in late 2021 or early 2022, depending on the timing of first patient treated in each study.

In parallel with pipeline advancement, the company has focused on establishing manufacturing and global distribution from clinical development through initial commercialization. Clinical supply for PBGM01 for our phase 1 / 2 global trial, manufactured through our partnership with Catalent, is already in place. We have also manufactured clinical supplies to initiate clinical trials for our next two most advanced programs – PBKRO3 for Krabbe disease and PBFT02 for FTD-GRN. In December, Passage Bio announced that it had completed construction and started production at its dedicated Current Good Manufacturing Practices (CGMP) manufacturing suite at Catalent Cell & Gene Therapy's facility in Maryland. The company also announced the planned opening in the second quarter of 2021 of a gene therapy manufacturing research and development site at the Princeton West Innovation Campus in Hopewell, New Jersey, for Chemistry, Manufacturing and Controls (CMC) laboratory operations to support analytics, assay development, and product testing for the company's gene therapy programs.

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 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 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:

Passage Bio Investors:
Sarah McCabe and Zofia Mita
Stern Investor Relations, Inc.
212-362-1200
sarah.mccabe@sternir.com
Zofia.mita@sternir.com

Passage Bio Media:
Gwen Fisher
Passage Bio
215-407-1548
gfisher@passagebio.com