U.S. Food and Drug Administration Grants Fast Track Designation to Three Passage Bio Gene Therapy Candidates Targeting Rare CNS Disorders

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FDA designation will facilitate development and expedited review of company's lead gene therapy product candidates for life-threatening diseases with unmet medical needs: PBGM01 for GM1 gangliosidosis (GM1), PBFT02 for frontotemporal dementia with granulin mutations, and PBKR03 for Krabbe disease

PHILADELPHIA, March 08, 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 announced the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to the company's three lead investigational gene therapies: PBGM01 for the treatment of GM1 gangliosidosis (GM1), PBFT02 for frontotemporal dementia with granulin mutations (FTD-GRN), and PBKR03 for Krabbe disease. Passage Bio plans to initiate clinical trials in GM1 in the first quarter of 2021 and in FTD-GRN and Krabbe disease in the first half of 2021.

"At Passage Bio, we are working to address some of the world's rarest and most devastating neurological diseases that affect infants and adults," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "The FDA's decision to grant Fast Track designation to each of our lead gene therapy candidates highlights the urgent need for new treatments in these diseases and represents an important step towards achieving our objective of getting potentially transformative therapies to patients as quickly as possible."

Fast Track designation facilitates the expedited development and review of a new drug that demonstrates potential to address unmet medical needs and treats a serious or life-threatening disease. Benefits of this designation include more frequent interactions with the FDA to discuss the drug's development plan, as well as eligibility for other regulatory mechanisms intended to expedite development and review, such as priority review.

"In the last year, we have laid the groundwork for Passage Bio to boldly execute on our strategy," said Dr. Goldsmith. "These regulatory designations are intended to accelerate the timelines in our efforts to make a meaningful difference in patients' lives."

PBGM01: Addressing the urgent need in infantile GM1

PBGM01 is being studied for the treatment of GM1, a rare and often life-threatening CNS disorder that impacts patients worldwide. Passage Bio is targeting the infantile form of the disease, which is the most severe, with a rapid disease course and no current treatment options beyond support care. PBGM01 has received Orphan Drug and Rare Pediatric Disease designations from the FDA, as well as Orphan Drug designation from the European Medicines Agency (EMA). Passage Bio has activated its first clinical trial site in the United States for the global Imagine-1 study evaluating PBGM01 and is currently recruiting patients. The company plans to dose the first patient in the trial in the first quarter of 2021 and to report initial 30-day safety and biomarker data mid-year.

More information about the global PBGM01 study, Imagine-1, can be found at ClinicalTrials.gov: NCT04713475

PBKR03: Potential one-time therapy for Krabbe disease

Passage Bio is developing PBKR03 to address the significant unmet treatment need in Krabbe disease, a rare pediatric disorder that has a devastating burden on patients and their families. The company plans to study PBKR03 as a monotherapy for the treatment of early infantile Krabbe disease and was previously granted both Orphan Drug and Rare Pediatric Disease designations for PBKR03 by the FDA. The Committee for Orphan Medicinal Products (COMP) at the EMA has also recently issued a positive opinion for an Orphan medicinal product designation for PBKR03. Final endorsement of the designation by the European Commission (EC) is expected later in the first quarter. Following FDA clearance of Passage Bio's Investigational New Drug (IND) application for PBKR03 in February 2021, the company plans to start a Phase 1/2 trial in the first half of 2021 and plans to report initial 30-day safety and biomarker data in late 2021 or early 2022.

More information about the global PBKR03 study, GALax-C, can be found at ClinicalTrials.gov: NCT04771416

PBFT02: Targeting the severe, life-threatening burden of FTD-GRN

Passage Bio is advancing clinical development of PBFT02 for treatment of patients with frontotemporal dementia with granulin mutations (FTD-GRN), a devastating form of early onset dementia. In addition to Fast Track designation, the FDA has granted PBFT02 Orphan Drug designation for the treatment of frontotemporal dementia. Passage Bio anticipates the start of the PBFT02 clinical program in the first half of 2021 with initial data readouts planned for late 2021 or early 2022.

More information about the global PBFT02 study, upliFT-D, can be found at ClinicalTrials.gov: NCT04747431

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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