Passage Bio's PBKR03 Receives Orphan Drug and Rare Pediatric Disease Designations from FDA for Treatment of Krabbe Disease

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PHILADELPHIA, Oct. 28, 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 U.S. Food and Drug Administration (FDA) has granted Orphan Drug and Rare Pediatric Disease (RPD) designations to PBKR03 for the treatment of Krabbe disease (Globoid Cell Leukodystrophy). Passage Bio expects to initiate a Phase 1/2 trial for PBKR03 in the first half of 2021. Krabbe disease is a rare and often life-threatening lysosomal storage disease that presents early in the patient's life, resulting in progressive damage to both the brain and peripheral nervous system.

"Receiving both Orphan Drug and Rare Pediatric Disease designations for PBKR03 underscore the urgent unmet medical need for children with Krabbe disease, for which there are no approved treatments," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "We are encouraged about the potential of PBKR03 as a life-altering therapy for this underserved patient population, and we look forward to working with the FDA as we solidify our plans to advance PBKR03 into clinical testing in 2021."

The FDA grants Orphan Drug designation to drugs and biologics intended for the treatment, diagnosis or prevention of rare diseases or conditions affecting fewer than 200,000 people in the United States. Orphan Drug designation affords Passage Bio the potential for certain benefits, including up to seven years of market exclusivity, assistance in the drug development process, tax credits for clinical development, and exemptions from certain FDA fees.

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, and subject to FDA approval of PBKR03 for the treatment of Krabbe disease, Passage Bio would be eligible to receive one priority review voucher, which could then be redeemed to receive priority review for any subsequent marketing application, or sold or transferred to other companies for their programs.

About Krabbe Disease

Krabbe disease is a rare and often life-threatening lysosomal storage disease caused by mutations in the GALC gene, which encodes galactosylceramidase, an enzyme that breaks down galactosylceramide and psychosine. Without adequate levels of galactosylceramidase, psychosine accumulates, causing widespread death of myelin-producing cells and progressive damage to nerves in both the brain and peripheral tissues. The early infantile form of the disease is the most severe and common, typically manifesting before six months of age and accounting for 60% to 70% of diagnoses. In these patients, the disease course is highly predictable and rapidly progresses to include loss of acquired milestones, staring episodes, apnea, peripheral neuropathy, severe weakness, unresponsiveness to stimuli, seizures, blindness, deafness and eventual death by two years of age. Late infantile patients, defined by onset between seven to twelve months of age, present similar symptoms and have a median survival of approximately five years from onset of symptoms. There are currently no disease-modifying therapies for Krabbe disease, and we believe incidence may be 2.6 in 100,000 births, which is higher than reported due to lack of adequate screening at birth.

About PBKR03

PBKR03 is an AAV-delivered gene therapy encoding GALC currently in late preclinical development for the treatment of infantile Krabbe disease, in which patients have mutations in the gene that codes for galactosylceramidase (GAL-C). Low GAL-C activity results in accumulation of psychosine which is toxic to the myelin producing oligodendrocytes of the CNS and Schwann cells in the periphery, resulting in damage to both the central and peripheral nervous systems. PBKR03 utilizes a next-generation proprietary AAVhu68 capsid to deliver, through intra-cisterna magna administration, a functional GALC gene. In preclinical models, PBKR03 has shown meaningful transduction of both the central and peripheral nervous system, with restoration of myelination in the brain and peripheral nerves. PBKR03 has the potential to treat both the central nervous system and peripheral nerve manifestations observed in Krabbe disease patients.

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.

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.

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