Passage Bio Announces Pipeline Expansion and Clinical Program Update

- *Exercised two additional options with University of Pennsylvania's Gene Therapy Program aligned with company's focus on CNS diseases with high unmet need*
    - *Huntington's disease, a fatal rare adult neurodegenerative disorder*
    - *Canavan disease, a fatal rare pediatric leukodystrophy*
- *Plan to report initial data from global Phase 1/2 trial for PBGM01 for treatment of infantile GM1 before year end*
- *Expect to dose the first patients in global Phase 1/2 trials for frontotemporal dementia and Krabbe disease in early 2022*

Philadelphia, PA – December 8, 2021 – Passage Bio, Inc. (Nasdaq: PASG), a clinical-stage genetic medicines company focused on developing transformative therapies for central nervous system disorders (CNS), today announced the expansion of its pipeline and provided a progress update on its three clinical programs.

"We are thrilled to announce the exercise of two additional options from our strategic research collaboration with Penn's Gene Therapy Program," said Bruce Goldsmith, Ph.D., president and chief executive officer. "Our two new programs in Huntington's disease and Canavan disease align with our mission to develop transformative CNS gene therapies for pediatric and adult patients with significant unmet medical need. We are excited to advance these programs and are focused on developing gene therapies that are well-differentiated with compelling value propositions."

"By virtue of the strength of our Gene Therapy Program collaboration, we now have three clinical-stage assets and a robust pipeline of six additional programs," Dr. Goldsmith added. "This gives us one of the leading genetic pipelines for CNS diseases in our industry. We look forward to sharing more about the progress of our pipeline early next year."

In addition to future updates early next year on the company's earlier-stage research, the company will report the initial safety and 30-day biomarker data for cohort 1 of its global Phase 1/2 clinical trial for PBGM01 for the treatment of infantile GM1 gangliosidosis (GM1) by year end. The company also expects to dose the first patients in the global Phase 1/2 trials for PBFT02 for frontotemporal dementia with granulin mutations and PBKR03 for Krabbe disease in early 2022. Passage Bio anticipates providing updated guidance on the timing of data readouts for these two studies after the first patients have been dosed.

"We are pleased with the continued expansion of our R&D partnership with Passage Bio in our joint quest to develop transformative therapies for patients with devastating CNS disorders," said James Wilson, M.D., Ph.D., director of Gene Therapy Program (GTP) at the University of Pennsylvania and chief scientific advisor of Passage Bio. "At GTP, we look forward to continuing to draw on our three decades of leadership in the pre-clinical development of AAV technologies to help identify optimal therapeutic candidates for Passage Bio to advance into the clinic."

About Passage Bio's Huntington Disease and Canavan Disease Programs

- **Huntington's Disease** – Passage Bio and GTP have initiated a pre-clinical research program to pursue in parallel multiple approaches for targeting the mutated huntingtin protein (HTT) and the unstable genetic repeat expansion to identify an optimal clinical candidate for the treatment of Huntington's disease. Currently, there are no approved disease-modifying therapies for Huntington's disease, a fatal rare genetic condition that involves the progressive degeneration of brain nerve cells, often affecting adults beginning in their 30s and 40s.

- **Canavan Disease** – Passage Bio and GTP have initiated a preclinical research program for Canavan disease, a rare and fatal pediatric leukodystrophy for which there are no approved disease-modifying therapies. The research involves evaluating several adeno-associated virus candidates to select one to advance into clinical development with a differentiated approach to restore the function of an enzyme called aspartoacylase (ASPA). The Canavan disease program complements Passage Bio's existing leukodystrophy pipeline portfolio, including PBGM01 for GM1 and PBKR03 for Krabbe disease.

About Huntington's and Canavan Disease

- **Huntington's disease** is caused by mutations in the HTT gene, which provides instructions for making a protein called huntingtin believed to play a physiological role in the brain. The symptoms of Huntington's disease include uncontrolled movements, emotional problems, and reduced abilities to think and conduct day-to-day activities. People with Huntington's disease usually die within 15 to 20 years of their diagnosis. Huntington's disease affects approximately one in 10,000 people, approximately 30,000 people in the United States. There are also an estimated 200,000 people in the United States at risk of developing the condition.

- With **Canavan disease**, the loss of function of ASPA impairs N-acetylaspartic acid metabolism, which leads to spongiform degeneration of white matter in the brain. Canavan disease is one of the most common degenerative cerebral diseases in infancy, with patients typically missing developmental milestones, having a rapidly increasing head circumference and progressively lacking motor control. They often do no live past their mid-teens. The incidence of Canavan disease is approximately one in 100,000 births worldwide, and is higher in people with Ashkenazi Jewish heritage, with approximately one in 6,400 to one in 13,500 births.

About Passage Bio

At Passage Bio (Nasdaq: PASG), we are on a mission to provide life-transforming genetic medicines for patients with 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.

Penn Financial Disclosure

Dr. Wilson is a Penn faculty member as well as a scientific collaborator, consultant and co-founder of Passage Bio. As such, he holds an equity stake in the company, receives sponsored research funding from Passage Bio, and as an inventor of certain Penn intellectual property that is licensed to Passage Bio, may receive additional financial benefits in the future. The University of Pennsylvania also receives sponsored research funding from Passage Bio and has licensed intellectual property to the company that may result in future financial returns to Penn.

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; and the ability of our lead product candidates to treat their respective target 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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