



## Passage Bio to Host First of a Series of 2021 Virtual R&D Events on January 25

January 19, 2021

PHILADELPHIA, Jan. 19, 2021 (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 it will host a series of virtual Research & Development events in 2021, with the first one scheduled for Monday, January 25, 2021, 11 a.m. to 1 p.m. ET.

The first event will focus on Passage Bio's lead investigational therapy PBGM01 and its disease target, infantile GM1 gangliosidosis (GM1). Presenters for the event are:

- **Bruce Goldsmith, Ph.D., chief executive officer**, who will present an overview of the Passage Bio pipeline and the GM1 disease and patient experience.
- **James M. Wilson, M.D., Ph.D., chief scientific advisor at Passage Bio and director of the Gene Therapy Program (GTP) at the University of Pennsylvania (Penn) and Christian Hinderer, M.D., Ph.D., senior research director at GTP at Penn**, who will review pre-clinical data supporting the clinical development of Passage Bio's PBGM01, as well as the scientific rationale for intra-cisterna magna delivery of the gene therapy.
- **Gary Romano, M.D., Ph.D., chief medical officer**, who will discuss the design for Imagine1, the PBGM01 global clinical program, as well as preliminary expectations for the trial.

To register for the live event, please use the following link: <https://www.webcaster4.com/Webcast/Page/359/39687>

A live webcast of the presentation will be available on the Investors & Media section of Passage Bio's website at [investors.passagebio.com](https://investors.passagebio.com) and will remain active for 30 days.

### About GM1

GM1, a rare monogenic lysosomal storage disease, is caused by mutations in the GLB1 gene, which encodes the lysosomal enzyme beta-galactosidase ( $\beta$ -gal). Reduced  $\beta$ -gal activity results in the accumulation of toxic levels of GM1 in neurons throughout the brain, causing rapidly progressive neurodegeneration. GM1 manifests with hypotonia (reduced muscle tone), progressive CNS dysfunction, and rapid developmental regression. Life expectancy for infants with GM1 is two to four years, and infantile GM1 represents approximately 60 percent of the global GM1 incidence of 0.5 to 1 in 100,000 live births.

### About PBGM01

PBGM01 is an AAV-delivery gene therapy currently being developed for the treatment of infantile GM1, in which patients have mutations in the GLB1 gene causing little or no residual  $\beta$ -gal enzyme activity and subsequent neurodegeneration. PBGM01 utilizes a next-generation AAVhu68 capsid administered through the intra-cisterna magna to deliver a functional GLB1 gene encoding  $\beta$ -gal to the brain and peripheral tissues. By reducing the accumulation of GM1 gangliosides, PBGM01 has the potential to reverse neuronal toxicity, thereby restoring developmental potential. In preclinical models, PBGM01 has demonstrated broad brain distribution and high levels of expression of the  $\beta$ -gal enzyme in both the CNS and critical peripheral organs, suggesting potential treatment for both the CNS and peripheral manifestations of GM1. PBGM01 has been granted Orphan Drug Designations by FDA and the European Commission as well as a Rare Pediatric Disease Designation by FDA.

### 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](https://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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