Passage Bio Reports Second Quarter 2020 Financial Results and Recent Business Highlights

August 13, 2020

– Submitted IND for PBGM01 Phase 1/2 trial; currently on FDA clinical hold pending additional review of proposed ICM delivery device –

– Anticipate dosing first patient in PBGM01 Phase 1/2 trial in late 4Q2020 or early 1Q2021 –

– Initial PBGM01 clinical safety and biomarker data remains on track for 1H2021 read out –

– Strong cash balance of $353M expected to fund operations into 2023 –

– Management to host conference call today at 8:30 a.m. ET –

PHILADELPHIA, Aug. 13, 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 reported financial results for the second quarter ended June 30, 2020 and provided recent business highlights.

"We have made substantial progress over the past quarter, including the submission of our first IND application to the FDA for a Phase 1/2 clinical trial in infantile GM1 patients with PBGM01, demonstrating our team's ability to work collaboratively with our partners at the Gene Therapy Program led by Dr. James Wilson," said Gary Romano, M.D., Ph.D, chief medical officer of Passage Bio. "We are confident that we can efficiently and successfully address the FDA clinical hold questions related to biocompatibility of our proposed ICM delivery device so that we can begin to dose patients before the end of this year or early next year. Importantly, we continue to believe that the initial clinical safety and biomarker data from this trial will be available late in the first half of 2021."

Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio, said: "I am proud of the world-class team we are building at Passage Bio, including substantial expansion of the clinical, manufacturing, and corporate operation teams. We have also been effective at navigating the unpredictable environment caused by COVID-19 both internally and with our key external partners to maintain our pipeline advancement. This has enabled us to make significant progress toward delivering on the promise of PBGM01 for patients suffering from GM1. We look forward to treating our first patient as well as demonstrating the potential of all our investigational product candidates as safe and effective treatment options for devastating rare diseases like GM1. With our operational progress and the continued strength of our balance sheet, together with our robust pipeline, we are well positioned to achieve these goals."

Recent Business Highlights

- **Received feedback from U.S. Food and Drug Administration (FDA) on Investigational New Drug (IND) application for PBGM01** – In June 2020, Passage Bio submitted its first ever IND for PBGM01 for the treatment of GM1 gangliosidosis to the FDA in collaboration with the University of Pennsylvania's Gene Therapy Program. Following this submission, the Company was notified that the IND was placed on clinical hold pending additional risk assessments of the biocompatibility of the proposed ICM delivery device. The ICM route of administration delivers PBGM01 directly to the brain into the cisterna magna, a space within the lower portion of the brain, with techniques and delivery devices commonly used both in current medical practice and other clinical trials, including those for gene therapy. The Company is evaluating options for conducting additional risk assessments while it awaits official written feedback from the FDA. During the IND review, the Company addressed specific clinical and protocol questions raised by the FDA, and the agency confirmed that there are no further clinical information requests. As a result of the clinical hold, the Company now expects to initiate dosing of its Phase 1/2 trial late in the fourth quarter of 2020 or early in the first quarter of 2021 and remains on track to report initial 30 day safety and biomarker data late in the first half of 2021.
- **Added to Russell 2000® Index** – In June 2020, Passage Bio was added to the Russell 2000® Index, a subset of the Russell 3000® Index, which measures the performance of the small-cap segment of the U.S. equity market.
- **Expanded gene therapy collaboration with the University of Pennsylvania (UPenn)** – In May 2020, Passage Bio expanded its research and development collaboration and licensing agreement with the University of Pennsylvania. The amendment increased the number of remaining available licensing options for programs to treat rare monogenic CNS disorders from six to eleven and extended the window for the exercise of options by three years. Accordingly, the window to exercise all eleven remaining options extends to May 2025. The Company also received exclusive rights and licenses, subject to limitations, to certain technologies resulting from discovery research at Gene Therapy Program (GTP) for Passage Bio products developed with GTP, such as novel capsids, toxicity reduction technologies and delivery and formulation improvements.
- **Announced presentation of preclinical data models of Krabbe Disease at American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting** – In May 2020, the University of Pennsylvania's Gene Therapy Program, Passage Bio's preclinical development partner, presented encouraging preclinical data from a Krabbe dog and Twitcher mouse model at the ASGCT Annual Meeting demonstrating the potential of an AAVhu68 capsid carrying a functional GALC gene to

normalize GALC enzyme activity and nerve conduction, leading to dose-dependent phenotypic correction and increased survival. Passage Bio is currently developing an AAVhu68 GALC therapeutic, PBKR03, for the treatment of infantile Krabbe disease and expects to initiate a Phase 1/2 trial in the first half of 2021.

- **Granted Orphan Drug and Rare Pediatric Disease designation by FDA for PBGM01** – In April and May 2020, respectively, the FDA granted Orphan Drug and Rare Pediatric Disease designation to PBGM01 for the treatment of infantile GM1. Orphan Drug designation is intended to promote the development of safe and effective treatments for rare diseases through certain financial and market exclusivity incentives, and Rare Pediatric Disease designation is intended to encourage treatments for life-threatening disease affecting children of 18 years of age or younger by qualifying the sponsor for a priority review voucher upon approval that may be sold or transferred.

Anticipated Upcoming Milestones

- Initiate a Phase 1/2 trial for the lead program, PBGM01, for the treatment of patients with infantile GM1 late in the fourth quarter of 2020 or early in the first quarter of 2021. Report initial 30-day safety and biomarker data late in the first half of 2021.
- Continue to advance lead programs PBFT02 for the treatment of frontotemporal dementia (FTD) and PBKR03 for the treatment of Krabbe disease toward clinical trial initiations in the first half of 2021.
- Continue to advance PBML04, PBLA05 and PBCM06 toward IND-enabling studies.

Second Quarter 2020 Financial Results

- **Cash Position:** Cash and cash equivalents were $353.4 million as of June 30, 2020 as compared to $158.9 million as of December 31, 2019.
- **Research and Development (R&D) Expenses:** R&D expenses were $19.9 million for the quarter ended June 30, 2020, compared to $6.3 million for the same quarter in 2019. The increase was primarily due to an increase of $4.5 million in costs incurred with the University of Pennsylvania in preparation for several IND filings, an increase of $4.0 million in clinical manufacturing costs, a $1.4 million increase in clinical development costs and a $0.5 million increase in consulting expense as we prepare for our clinical trials to begin in the second half of 2020 and early 2021. The Company also had a $3.1 million increase in personnel-related costs and a $0.1 million increase in facility and other costs due to increases in employee headcount in the R&D function.
- **General and Administrative (G&A) Expenses**: G&A expenses were $7.4 million for the quarter ended June 30, 2020, compared to $1.0 million for the same quarter in 2019. The increase was primarily due to a $4.7 million increase in personnel-related and share-based compensation expense due to increases in employee headcount. The Company's professional fees and facility costs also increased by $0.7 million and $1.0 million, respectively, as Passage Bio expanded its operations to support its R&D efforts.
- **Net Loss**: Net loss was $27.2 million, or a net loss of $0.60 per basic and diluted share, for the quarter ended June 30, 2020, compared to $13.4 million, or a net loss of $3.19 per basic and diluted share, for the quarter ended June 30, 2019.

Conference Call Details

Passage Bio will host a conference call and webcast today at 8:30 a.m. ET. To access the live conference call, please dial 833-528-0605 (domestic) or 830-221-9711 (international) and reference conference ID number 5679946. A live audio webcast of the event will be available on the Investors & Media section of Passage Bio's website at investors.passagebio.com. The archived webcast will be available on Passage Bio's website approximately two hours after the completion of the event and for 30 days following the call.

About Passage Bio

Passage Bio is a genetic medicines company focused on developing transformative therapies for rare, monogenic central nervous system disorders with limited or no approved treatment options. The company is based in Philadelphia, PA and has a research, collaboration and license agreement with the University of Pennsylvania and its Gene Therapy Program (GTP). The GTP conducts discovery and IND-enabling preclinical work and Passage Bio conducts all clinical development, regulatory strategy and commercialization activities under the agreement. The company has a development portfolio of six product candidates, with the option to license eleven more, with lead programs in GM1 gangliosidosis, frontotemporal dementia and Krabbe disease.

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, resolution of the clinical hold on PBGM01, initiation of clinical trials and the availability of clinical data from such trials; our cash forecasts, our expectations about our collaborators' and partners' ability to execute key initiatives; 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, obtain regulatory approval for and commercialize our product candidates; the timing and results of preclinical studies and clinical trials; 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; 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; failure to protect and enforce our intellectual property, and other proprietary

rights; failure to successfully execute or realize the anticipated benefits of our strategic and growth initiatives; risks relating to technology failures or breaches; our dependence on collaborators and other third parties for the development 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 COVID-19 pandemic; risks associated with current and potential future healthcare reforms; risks relating to attracting and retaining key personnel; failure to comply with legal and regulatory requirements; risks relating to access to capital and credit markets; 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.

Passage Bio, Inc.
Balance Sheets
(unaudited)

(in thousands, except share data)	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 353,423	$ 158,874
Prepaid expenses	2,176	156
Prepaid research and development	12,631	6,745
Total current assets	368,230	165,775
Property and equipment, net	1,096	1,087
Other assets	8,771	11,751
Total assets	$ 378,097	$ 178,613
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 7,214	$ 629
Accrued expenses and other current liabilities	6,920	3,052
Total current liabilities	14,134	3,681
Deferred rent	524	504
Other liabilities	43	76
Total liabilities	14,701	4,261
Convertible preferred stock, $0.0001 par value:		
Series A-1 convertible preferred stock: No shares authorized, issued and outstanding at March 31, 2020; 63,023,258 shares authorized, issued and outstanding at December 31, 2019	-	74,397
Series A-2 convertible preferred stock: No shares authorized, issued and outstanding at March 31, 2020; 22,209,301 shares authorized; issued and outstanding at December 31, 2019	-	46,311
Series B convertible preferred stock: No shares authorized, issued and outstanding at March 31, 2020; 33,592,907 shares authorized, issued and outstanding at December 31, 2019	-	109,897
Total convertible preferred stock	-	230,605
Stockholders' equity (deficit) :		
Common stock, $0.0001 par value: 100,000,000 shares authorized; 45,797,195 shares issued and 45,350,687 shares outstanding at March 31, 2020 and 5,194,518 shares issued and 4,293,039 shares outstanding at December 31, 2019	4	-
Additional paid-in capital	466,812	2,410
Accumulated deficit	(103,420)	(58,663)
Total stockholders' equity (deficit)	363,396	(56,253)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 378,097	$ 178,613

Statements of Operations
(unaudited)

(in thousands, except share and per share data)	Three Months Ended June 30, 2020	Three Months Ended June 30, 2019	Six Months Ended June 30, 2020	Six Months Ended June 30, 2019
Operating expenses:				
Research and development	$ 19,902	$ 6,299	$ 33,019	$ 9,332
Acquired in-process research and development	-	500	-	500
General and administrative	7,402	968	12,197	2,122
Loss from operations	(27,304)	(7,767)	(45,216)	(11,954)

Change in fair value of future tranche right liability	-	(5,659)	-	(9,141)
Interest income	132	-	459	-
Net loss	$ (27,172)	$ (13,426)	$ (44,757)	$ (21,095)
Per share information:				
Net loss per share of common stock, basic and diluted	$ (0.60)	$ (3.19)	$ (1.42)	$ (5.02)
Weighted average common shares outstanding, basic and diluted	45,386,308	4,209,716	31,581,851	4,203,694

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