



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

May 11, 2020

- *Expansion of gene therapy collaboration with University of Pennsylvania's Gene Therapy Program extends collaboration by three additional years and adds five new programs –*
- *Orphan Drug Designation recently granted by FDA for lead development candidate PBGM01; program expected to enter the clinic for treatment of infantile GM1 in fourth quarter of 2020 –*
- *Strong cash balance of \$367M expected to fund operations into 2023 –*

PHILADELPHIA, May 11, 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 first quarter ended March 31, 2020 and provided recent business highlights.

"The first quarter was a significant time for Passage Bio and the patients that we serve. Building upon the strong momentum since launching the company in 2018, we completed an upsized IPO in March 2020 and are now working to bring our lead program, PBGM01, for the treatment of infantile GM1 gangliosidosis into the clinic as soon as possible and advancing the balance of our deep pipeline," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "While the potential impacts of the COVID-19 pandemic are uncertain, with our deeply experienced management team, supported by a strong cash position, we are confident in our ability to continue to execute and remain on-track to meet our 2020 clinical development goals. As a company that aims, above all, to serve patients and families suffering from rare, life-threatening CNS diseases with no alternative treatment options, we are committed to driving our programs forward."

Recent Business Highlights

- **Expansion of gene therapy collaboration with the University of Pennsylvania (UPenn)** – In May 2020, Passage Bio expanded its research and development collaboration and licensing agreement with UPenn. 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.
- **Granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the lead product candidate PBGM01** – In April 2020, the FDA granted ODD to PBGM01 for the treatment of infantile GM1 gangliosidosis (GM1), a rare and often life-threatening monogenic recessive lysosomal storage disease caused by mutations in the GLB1 gene that results in rapidly progressing neurodegeneration. The designation grants the company certain benefits, including financial incentives to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.
- **Completed upsized Initial Public Offering (IPO)** – In March 2020, Passage Bio completed its IPO of 13,800,000 shares of common stock at a public offering price of \$18.00 per share, including an exercise of the underwriter's option to purchase additional shares. The total net proceeds from the offering, after deducting underwriting discounts, commissions and offering expenses, were \$227.5 million.
- **Appointed Bruce Goldsmith, Ph.D. as president and chief executive officer** – In January 2020, Passage Bio announced the appointment of Bruce Goldsmith, Ph. D., as president and chief executive officer, succeeding co-founder and interim chief executive officer Stephen Squinto, Ph.D., who now serves as acting head of research and development for the company.
- **Strengthened company's Board of Directors (BOD) with additions of Sandip Kapadia and Athena Countouriotis, M.D.** – In January and March 2020 respectively, Passage Bio appointed Sandip Kapadia, MBA, CPA, and Athena Countouriotis, M.D., to its BOD. Mr. Kapadia currently serves as the chief financial officer and treasurer of Intercept Pharmaceuticals, and Dr. Countouriotis is currently president and chief executive officer at Turning Point Therapeutics.

Anticipated Upcoming Milestones

- Initiate a Phase 1/2 trial for the lead program, PBGM01, for the treatment of patients with infantile GM1 in fourth quarter of 2020.
- Continue to advance the lead programs PBFT02, for the treatment of frontotemporal dementia (FTD) and PBKR03, for the treatment of Krabbe disease toward clinical study initiation in the first half of 2021.
- Continue to advance PBML04, PBLA05 and PBCM06 toward Investigational New Drug (IND)-enabling studies.

First Quarter 2020 Financial Results

- **Cash Position:** Cash and cash equivalents were \$366.8 million as of March 31, 2020 as compared to \$158.9 million as of December 31, 2019. In February 2020, Passage Bio raised \$227.5 million in net proceeds from its IPO.
- **Research and Development (R&D) Expenses:** R&D expenses were \$13.1 million for the quarter ended March 31, 2020, compared to \$3.0 million for the quarter ended March 31, 2019. The increase was primarily due to an increase of \$4.8 million in R&D costs incurred with Penn and were related to the preparation of several IND filings as well as an increase in other research costs of \$2.9 million as the company prepares for its clinical trials to begin in the second half of 2020 and early 2021. The company also had a \$2.3 million increase in personnel-related costs and a \$0.2 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 \$4.8 million for the quarter ended March 31, 2020, compared to \$1.2 million for the quarter ended March 31, 2019. The increase was primarily due to a \$2.2 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 \$0.6 million and \$0.8 million, respectively, as Passage Bio expanded its operations to support its R&D efforts.
- **Net Loss:** Net loss was \$17.6 million, or a net loss of \$1.00 per basic and diluted share, for the quarter ended March 31, 2020, compared to \$7.7 million, or a net loss of \$1.83 per basic and diluted share, for the quarter ended March 31, 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 2588609. 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, 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 Sheet

(in thousands, except share data)	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 366,828	\$ 158,874
Prepaid expenses	2,921	156
Prepaid research and development	12,340	6,745
Total current assets	382,089	165,775
Property and equipment, net	1,137	1,087

Other assets	9,201	11,751
Total assets	<u>\$ 392,427</u>	<u>\$ 178,613</u>
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	\$ 2,532	\$ 629
Accrued expenses and other current liabilities	2,696	3,052
Total current liabilities	5,228	3,681
Deferred rent	489	504
Other liabilities	44	76
Total liabilities	<u>5,761</u>	<u>4,261</u>
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	<u>-</u>	<u>230,605</u>
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	462,910	2,410
Accumulated deficit	(76,248)	(58,663)
Total stockholders' equity (deficit)	<u>386,666</u>	<u>(56,253)</u>
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 392,427</u>	<u>\$ 178,613</u>

**Statements of Operations
(unaudited)**

(in thousands, except share and per share data)	Three Months Ended March 31,	
	2020	2019
Operating expenses:		
Research and development	\$ 13,117	\$ 3,033
General and administrative	4,795	1,154
Loss from operations	(17,912)	(4,187)
Change in fair value of future tranche right liability	-	(3,482)
Interest income	327	-
Net loss	<u>\$ (17,585)</u>	<u>\$ (7,669)</u>
Per share information:		
Net loss per share of common stock, basic and diluted	\$ (1.00)	\$ (1.83)
Weighted average common shares outstanding, basic and diluted	<u>17,624,011</u>	<u>4,197,604</u>

For further information, please contact:

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