Passage Bio Reports First Quarter 2021 Financial Results and Recent Business Highlights

May 5, 2021

- *Dosed first patient with infantile GM1 gangliosidosis in Imagine-1 Phase 1/2 trial of PBGM01, initial safety and 30-day biomarker data expected 4Q21*
- *Received multiple regulatory clearances for clinical trial initiations for three most advanced pipeline programs*
- *Expected enrollment of first patient in Phase 1/2 FTD-GRN trial in 2Q/3Q21 and first patient in Phase 1/2 Krabbe trial in 3Q21*
- *Continued to advance pipeline under leadership of newly appointed Chief R&D Officer Eliseo Salinas, M.D., MSc.*
- *Appointed Maxine Gowen, Ph.D., an experienced biotech leader, to Passage Bio Board of Directors*
- *Raised $166 million, further strengthening company's cash position*
- *Management to host conference call today at 8:30 a.m. ET*

PHILADELPHIA, May 05, 2021 (GLOBE NEWSWIRE) -- Passage Bio, Inc. (Nasdaq: PASG), a clinical-stage 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, 2021 and provided recent business highlights.

"We are particularly proud to have recently dosed our first patient in our global Imagine-1 trial of PBGM01 for infantile GM1 gangliosidosis," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "This accomplishment speaks to the strength of our collaboration with the University of Pennsylvania's Gene Therapy Program. By raising an additional $166 million in the first quarter, we are well funded to advance both our clinical- and research-stage programs.

"Our primary focus in 2021 remains advancing and expanding our differentiated pipeline for patients with rare CNS disorders," Dr. Goldsmith added. "We are pleased to have successfully received a number of clinical trial regulatory approvals in a period of several months for our three most advanced programs. While some impact on clinical site initiations from the Covid-19 pandemic has occurred, which is reflected in adjusted study timelines, we look forward to reporting on several meaningful milestones throughout 2021 as we diligently work toward delivering transformative CNS therapies for patients."

Recent Highlights:

- **First patient dosed in Imagine-1 study of PBGM01 for infantile GM1 gangliosidosis:** The company announced in April 2021 that the first patient had been dosed in its Imagine-1 global Phase 1/2 trial of PBGM01 for the treatment of infantile GM1 gangliosidosis (GM1). Imagine-1 is a global open-label study of PBGM01 administered by a single injection into the cisterna magna in pediatric subjects with early and late infantile GM1.

- **Several recent regulatory milestones achieved in a period of three months for three most advanced pipeline programs:**
  - **PBGM01** for GM1 and **PBKR03** for Krabbe disease received regulatory clearances for clinical trial initiations from U.S. Food and Drug Administration (FDA), UK MHRA, and Health Canada
  - **PBGM01** also received regulatory clearance for clinical trial initiation from the Brazilian Regulatory Health Agency
  - **PBFT02** for frontotemporal dementia with granulin mutations (FTD-GRN) received regulatory clearances for clinical trial initiation from FDA and Health Canada
  - The European Commission granted Orphan designation for **PBKR03** for the treatment of early infantile Krabbe disease.

- **Announced collaboration with InformedDNA, the nation's leading genetics services organization:** The collaboration will provide no-cost genetic counseling and testing for adults who have been diagnosed with FTD. The testing program will facilitate identification of FTD patients with certain inherited genetic mutations as well as support for clinical trial recruitment and enrollment. It is estimated that approximately 5 to 10 percent of FTD is caused by a GRN gene mutation.

- **Timelines adjusted for global Phase 1/2 clinical trials for PBGM01, PBFT02 and PBKR03 due primarily to impacts associated with Covid-19:** The initial safety and 30-day biomarker data for Imagine-1 for PBGM01 for GM1 is expected to read out in 4Q21**.** The global clinical trial upliFT-D for PBFT02 for FTD-GRN is expected to initiate in 2Q/3Q 2021 with initial safety and 30-day biomarker data to be reported in 1H22. The global clinical trial GALax-C for PBKR03 for Krabbe disease is expected to initiate in 3Q21 with initial safety and 30-day biomarker data to be reported in 1H22.

- **Eliseo O. Salinas, M.D., MSc, appointed as chief research & development officer:** In March 2021, the company

appointed Dr. Salinas as the company's chief research and development (R&D) officer. Dr. Salinas has extensive experience in the development of small molecules, biologics and cell therapy and has been directly involved with numerous investigational new drug (IND) application submissions and regulatory approvals in the United States and globally.

- **Maxine Gowen, Ph.D., appointed to Passage Bio board of directors:** In February 2021, the company announced the appointment of Dr. Gowen to its board of directors. She brings significant public company leadership and clinical development expertise, having co-founded and led a start-up biotech company and served in a variety of leadership roles at GlaxoSmithKline over a period of 15 years.

- **Raised $166 million in public offering of common stock:** In January 2021, the company announced a public offering of 7,000,000 shares of its common stock at a price of $22 per share. The underwriters also exercised their option to purchase an additional 1,050,000 shares of common stock for total offering net proceeds of $166 million.

Anticipated Upcoming Milestones

- Host R&D day focused on FTD-GRN, May 17.
- Open CMC research and development site in Hopewell, NJ, by the end of the second quarter of 2021.
- Report initial safety and 30-day biomarker data from Phase 1/2 PBGM01 trial in 4Q21.
- Dose first patient in Phase 1/2 FTD-GRN trial in 2Q/3Q21; report initial safety and 30-day biomarker data in 1H22.
- Dose first patient in Phase 1/2 Krabbe trial in 3Q21; report initial safety and 30-day biomarker data in 1H22.
- Continue to advance preclinical programs for PBML04 (Metachromatic leukodystrophy), PBLA05 (Amyotrophic lateral sclerosis) and PBCM06 (Charcot-Marie-Tooth Disease Type 2A), and an undisclosed adult CNS program.

First Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $437.6 million as of March 31, 2021 as compared to $304.8 million as of December 31, 2020. This includes $166 million in net proceeds from the company's public offering in January 2021.
- **Research and Development (R&D) Expenses:** R&D expenses were $25.0 million for the first quarter ended March 31, 2021, compared to $13.1 million for the same quarter in 2020. Acquired in-process R&D expenses were $1.5 million for the first quarter ended March 31, 2021, compared to none in the same quarter of 2020.
- **General and Administrative (G&A) Expenses**: G&A expenses were $12.5 million for the first quarter ended March 31, 2021, compared to $4.8 million for the same quarter in 2020.
- **Net Loss**: Net loss was $38.9 million, or a net loss of $0.76 per basic and diluted share, for the quarter ended March 31, 2021 compared to $17.6 million, or a net loss of $1.00 per basic and diluted share, for the same quarter in 2020.

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 6366724. 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
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 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.

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; our expectations about manufacturing plans and strategies; our expectations about cash runway; and the ability of our lead product candidates to treat 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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Passage Bio, Inc.
Balance Sheets
(Unaudited)

(in thousands, except share data)	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 249,521	$ 135,002
Marketable securities	188,068	169,815
Prepaid expenses	3,374	1,405
Prepaid research and development	9,986	10,961
Total current assets	450,949	317,183
Property and equipment, net	6,574	2,795
Other assets	7,649	8,029
Total assets	$ 465,172	$ 328,007
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 6,248	$ 5,265
Accrued expenses and other current liabilities	13,221	15,910
Total current liabilities	19,469	21,175
Deferred rent	4,199	2,077
Other liabilities	—	41
Total liabilities	23,668	23,293
Commitments and Contingencies		
Stockholders' equity:		
Common stock, $0.0001 par value: 300,000,000 shares authorized; 53,977,484 shares issued and 53,848,324 shares outstanding at March 31, 2021 and 45,917,084 shares issued and 45,614,807 shares outstanding at December 31, 2020	5	4
Additional paid-in capital	651,283	475,617
Accumulated other comprehensive loss	(7)	(12)
Accumulated deficit	(209,777)	(170,895)
Total stockholders' equity	441,504	304,714
Total liabilities and stockholders' equity	$ 465,172	$ 328,007

Passage Bio, Inc.
Statements of Operations and Comprehensive Loss
(Unaudited)

	Three Months Ended March 31,	
(in thousands, except share and per share data)	**2021**	**2020**
Operating expenses:		
Research and development	$ 24,970	$ 13,117

Acquired in-process research and development	1,500	—
General and administrative	12,464	4,795
Loss from operations	(38,934)	(17,912)
Interest income, net	52	327
Net loss	$ (38,882)	$ (17,585)
Per share information:		
Net loss per share of common stock, basic and diluted	$ (0.76)	$ (1.00)
Weighted average common shares outstanding, basic and diluted	51,331,449	17,624,011
Comprehensive loss:		
Net loss	$ (38,882)	$ (17,585)
Unrealized gain on marketable securities	5	—
Comprehensive loss	$ (38,877)	$ (17,585)