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

November 10, 2020

– Dosing for first patient in PBGM01 Phase 1/2 trial anticipated in 1Q2021; initial safety and biomarker data expected mid-year 2021 –
– Phase 1/2 clinical trials for FTD-GRN and Krabbe expected to initiate in 1H21 –
– Completion of dedicated manufacturing suite achieved to ensure control of supply of company's lead products through early commercialization –
– Management to host conference call today at 8:30 a.m. ET –

PHILADELPHIA, Nov. 10, 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 third quarter ended September 30, 2020 and provided recent business highlights.

"Over the past quarter, we have made important strides in strengthening our operations to support the initiation of three Phase 1/2 clinical trials for our lead programs in infantile GM1, FTD-GRN, and Krabbe disease in the first half of 2021," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "We are particularly pleased with our progress in terms of patient identification, clinical trial site preparedness and manufacturing readiness. We also remain confident that we will receive FDA clearance for our investigational new drug application for PBGM01 for the treatment of infantile GM1. This has all been made possible because of the highly experienced, talented team that we have assembled over the past year to be ready to execute seamlessly in 2021 on our path to providing life-transforming gene therapies for patients with rare, monogenic CNS disorders."

Recent Corporate Highlights

- **Work progresses in anticipation of U.S. Food and Drug Administration (FDA) clearance for investigational new drug (IND) application for PBGM01:** In August 2020, Passage Bio received its official letter from FDA confirming that the clinical hold of its IND for PBGM01 for the treatment of infantile GM1 gangliosidosis (GM1) was due solely to questions concerning the biocompatibility of the proposed intra cisterna magna (ICM) delivery device. Passage Bio is working diligently to resolve the clinical hold and, based on feedback from FDA, is conducting biocompatibility risk assessments and testing of the ICM device. The company remains confident that the IND will be cleared. As a result of these efforts to obtain FDA clearance of the device, Passage Bio now plans to initiate its Phase 1/2 trial in the first quarter of 2021 and to report initial 30-day safety and biomarker data mid-year 2021.
- **Dedicated GMP manufacturing suite is complete:** Construction and qualification of Passage Bio's dedicated suite at Catalent are complete. With this suite, the company will now be able to provide clinical supplies for its lead pipeline products through early commercialization and expects to initiate manufacturing activities in the coming months. Clinical supply for the GM1 Phase 1/2 trial is already in place through Passage Bio's ongoing partnership with Catalent.
- **Orphan Drug Designation (ODD) granted by European Commission (EC) for PBGM01 –** In October 2020, the EC granted ODD to PBGM01 for the treatment of GM1, a rare and often life-threatening monogenic recessive lysosomal storage disease caused by mutations in the GLB1 gene, resulting in rapidly progressing neurodegeneration. PBGM01 has previously been granted ODD and RPDD by FDA for the treatment of GM1.
- **Rare Pediatric Disease Designation (RPDD) and ODD granted by FDA for PBKR03 –** In October 2020, FDA granted RPDD and ODD to PBKR03 for the treatment of Krabbe disease, a rare and often life-threatening lysosomal storage disease caused by mutations in the GALC gene resulting in progressive damage to both the brain and peripheral nervous system.
- **Collaboration with Invitae in place to facilitate genetic testing and support diagnosis of patients with GM1 –** Passage Bio recently announced it has entered into a collaboration with Invitae, a leading medical genetic testing company, to offer free genetic testing and counselling for GM1 through Invitae's Detect Lysosomal Storage Disorders program to encourage early diagnosis. As part of the collaboration, Invitae will also provide educational information to patients and clinicians regarding clinical trials.
- **Recently published preclinical data in peer-reviewed journals support advancement into the clinic of PBGM01 and PBFT02 –** Passage Bio earlier announced the publications of preclinical data from the University of Pennsylvania's Gene Therapy Program supporting the potential of PBGM01 and PBFT02 to correct the underlying genetic defect associated with GM1 and frontotemporal dementia (FTD) with a granulin (GRN) mutation, respectively. Data from a mouse model of GM1 demonstrated that a single intracerebroventricular injection of an optimized adeno-associated virus (AAV) into the cerebral spinal fluid (CSF) resulted in significant expression of β-gal in the brain and peripheral tissues, and demonstrated dose-related reductions in neuronal lysosomal storage lesions, reduced neurological impairment and improvement in survival. Data from the FTD mouse model showed a single administration of an AAV containing the GRN gene resulted in elevated levels of progranulin in the brain and CSF, reduced lysosomal storage lesions, normalized lysosomal enzyme expression and corrected microgliosis. These GM1 data were published in *Human Gene Therapy*, and FTD-GRN data

were published in *Annals of Clinical and Translational Neurology*.

Anticipated Upcoming Milestones

- Initiate Phase 1/2 trial for the Company's lead program, PBGM01, for the treatment of patients with infantile GM1 in the first quarter of 2021. Report initial 30-day safety and biomarker data mid-year 2021.
- Continue to advance lead programs PBFT02 for the treatment of FTD-GRN and PBKR03 for the treatment of Krabbe disease toward Phase 1/2 clinical trial initiations in the first half of 2021.
- Continue to advance pre-clinical programs for PBML04 (Metachromatic leukodystrophy), PBLA05 (Amyotrophic lateral sclerosis) and PBCM06 (Charcot-Marie-Tooth Disease Type 2A).

Third Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $335.7 million as of September 30, 2020 as compared to $158.9 million as of December 31, 2019.
- **Research and Development (R&D) Expenses:** R&D expenses were $20.8 million for the quarter ended September 30, 2020, compared to $10.4 million for the same quarter in 2019.
- **General and Administrative (G&A) Expenses**: G&A expenses were $7.8 million for the quarter ended September 30, 2020, compared to $1.2 million for the same quarter in 2019.
- **Net Loss**: Net loss was $28.5 million, or a net loss of $0.63 per basic and diluted share, for the quarter ended September 30, 2020, compared to $11.4 million, or a net loss of $2.68 per basic and diluted share, for the same quarter in 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

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 unparalleled 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 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.

Passage Bio, Inc.
Balance Sheets
(unaudited)

(in thousands, except share data)	September 30, 2020	December 31, 2019
Assets		
Current assets:		

Cash and cash equivalents	$ 200,570	$ 158,874
Marketable securities	135,131	—
Prepaid expenses	1,748	156
Prepaid research and development	12,058	6,745
Total current assets	349,507	165,775
Property and equipment, net	915	1,087
Other assets	8,269	11,751
Total assets	$ 358,691	$ 178,613
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 3,441	$ 629
Accrued expenses and other current liabilities	15,662	3,052
Total current liabilities	19,103	3,681
Deferred rent	643	504
Other liabilities	42	76
Total liabilities	19,788	4,261
Convertible preferred stock, $0.0001 par value:		
Series A-1 convertible preferred stock: 63,023,258 shares authorized, issued and outstanding at December 31, 2019	—	74,397
Series A-2 convertible preferred stock: 22,209,301 shares authorized; issued and outstanding at December 31, 2019	—	46,311
Series B convertible preferred stock: 33,592,907 shares authorized, issued and outstanding at December 31, 2019	—	109,897
Total convertible preferred stock	—	230,605
Commitments and Contingencies (note 7)		
Stockholders' equity (deficit):		
Common stock, $0.0001 par value: 300,000,000 shares authorized; 45,885,052 shares issued and 45,543,682 shares outstanding at September 30, 2020 and 5,194,518 shares issued and 4,293,039 shares outstanding at December 31, 2019	4	—
Additional paid-in capital	470,890	2,410
Accumulated other comprehensive loss	(40)	—
Accumulated deficit	(131,951)	(58,663)
Total stockholders' equity (deficit)	338,903	(56,253)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 358,691	$ 178,613

Statements of Operations
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except share and per share data)	**2020**	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 20,837	$ 10,434	$ 53,856	$ 19,766
Acquired in-process research and development	—	—	—	500
General and administrative	7,793	1,209	19,990	3,331
Loss from operations	(28,630)	(11,643)	(73,846)	(23,597)
Change in fair value of future tranche right liability	—	—	—	(9,141)
Interest income, net	99	255	558	255
Net loss	$ (28,531)	$ (11,388)	$ (73,288)	$ (32,483)
Per share information:				
Net loss per share of common stock, basic and diluted	$ (0.63)	$ (2.68)	$ (2.02)	$ (7.70)
Weighted average common shares outstanding, basic and diluted	45,503,794	4,248,835	36,273,495	4,218,907
Comprehensive loss:				
Net loss	$ (28,531)	$ (11,388)	$ (73,288)	$ (32,483)
Unrealized loss on available-for-sale investments	(40)	—	(40)	—
Comprehensive loss	$ (28,571)	$ (11,388)	$ (73,328)	$ (32,483)

For further information, please contact:

Investors:
Sarah McCabe and Zofia Mita
Stern Investor Relations, Inc.
sarah.mccabe@sternir.com
zofia.mita@sternir.com

Media:
Gwen Fisher
Passage Bio
215.407.1548
gfisher@passagebio.com