Passage Bio Reports Fourth Quarter and Full-Year 2020 Financial Results and Recent Business Highlights

March 3, 2021

- On track to initiate three Phase 1/2 clinical programs in the first half of 2021: PBGM01 for GM1 gangliosidosis (GM1), PBFT02 for frontotemporal dementia with granulin mutations, and PBKR03 for Krabbe disease -
- Opened first U.S. site for PBGM01's Imagine-1 global clinical program and actively recruiting patients; other site activations in progress -
- Opening new CMC research and development site to support analytics, assay development, and product testing in 2Q 2021 -
- Strengthened financial position with recent public offering that raised $166M in net proceeds -
- Management to host conference call today at 8:30 a.m. ET -

PHILADELPHIA, March 03, 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 reported financial results for the fourth quarter and full year ended December 31, 2020 and provided recent business highlights.

"At Passage Bio, we are on a mission to provide life-transforming therapies to patients with devastating CNS diseases, and we are pleased with the foundational work we accomplished in 2020 as we transition to a clinical-stage company in 2021," said Bruce Goldsmith, Ph.D., president and chief executive officer of Passage Bio. "In the year ahead, our priorities are progressing three clinical development programs, all set to begin in the first half of 2021, and continuing to expand our pipeline and internal operations. We are confident that with the expertise of our talented internal team, our partnership with University of Pennsylvania's Gene Therapy Program, and our relationships with patient and physician groups that we are well positioned for strong execution across all our upcoming milestones."

Recent Corporate Highlights

- **On track to dose first patient in Imagine-1 trial evaluating PBGM01 for the treatment of infantile GM1 gangliosidosis:** In January 2021, the company announced that U.S. Food and Drug Administration (FDA) cleared its Investigational New Drug (IND) application for PBGM01 for the treatment of infantile GM1 gangliosidosis (GM1), a rare and often life-threatening CNS disorder with no approved disease-modifying therapies. The company has activated its first site in the United States and is currently recruiting patients. PGBM01 has also received regulatory clearance to begin studies in the United Kingdom and Canada. Site activation efforts are in progress in these countries as well as for additional sites in the United States. The company continues to expect to dose the first patient in the trial in first quarter of 2021 and to report initial 30-day safety and biomarker data mid-year 2021. The investigational therapy has also received orphan drug and rare pediatric disease designations from FDA, as well as an orphan drug designation from the European Medicines Agency for the treatment of GM1.

- **IND cleared and preparations on track to initiate Phase 1/2 trial for PBKR03 for treatment of patients with Krabbe disease, a rare pediatric disease:** The company announced FDA clearance of the IND application for PBKR03 for Krabbe disease, a rare lysosomal storage disease, in February 2021. PBKR03 was also previously granted both orphan drug and rare pediatric disease designations for the treatment of Krabbe disease by FDA. The European Medicines Agency has also recently adopted a positive opinion for an orphan drug designation for PBKR03. Final endorsement of the designation by the European Commission is expected later in the first quarter. Passage Bio anticipates the start of the PBKR03 clinical program in the first half of 2021 with an initial 30-day safety and biomarker data readout planned for late 2021 or early 2022.

- **IND cleared and preparations on track to initiate Phase 1/2 trial for PBFT02 for treatment of patients with frontotemporal dementia with granulin mutations (FTD-GRN), an adult indication:** In January, the company announced that FDA cleared its IND application for PBFT02 for FTD-GRN, a devastating form of early onset dementia. FDA has recently granted fast track designation for PBFT02. Fast track is a process designed to facilitate the development and expedite the review of drugs to treat conditions and fill unmet medical need. PBFT02 was also granted orphan drug designation by FDA earlier this year for the treatment of FTD-GRN. Passage Bio anticipates the start of PBFT02 clinical program in the first half of 2021 with initial data readouts planned late 2021 or early 2022.

- **Raised $166M in net proceeds via a 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 a total offering net proceeds of $166 million.

- **Continue to license and advance assets through partnership with the University of Pennsylvania (Penn)'s Gene**

Therapy Program (GTP): The company recently advanced PBML04 for the treatment of metachromatic leukodystrophy (MLD) into IND-enabling studies in partnership with Penn GTP. Through its partnership with GTP, the company also licensed a seventh unnamed program for the treatment of an adult neurodegenerative rare monogenic CNS disorder. Passage Bio has licensing options for a total of 17 gene therapy research programs focused on rare monogenic CNS disorders and continues to evaluate additional programs for further development.

- **Establishing internal and external manufacturing capabilities to meet the needs of growing pipeline**: In December 2020, Passage Bio announced that it had completed construction and initiated vector manufacturing at its dedicated Current Good Manufacturing Practices (CGMP) manufacturing suite at Catalent Cell & Gene Therapy's facility in Maryland. The company also announced its plans to open a Chemistry, Manufacturing and Controls (CMC) research and development site at the Princeton West Innovation Campus in Hopewell, New Jersey, for CMC laboratory operations to support analytics, assay development, and product testing for the company's gene therapy programs in the second quarter of 2021. The company has completed clinical supply and established global clinical distribution to support clinical development of PBGM01 as well manufactured clinical supplies to initiate clinical trials for PBFT02 for FTD-GRN and PBKR03 for Krabbe disease.

- **Strengthened patient identification and engagement efforts, including**:

    - Sponsorship of ScreenPlus pilot program, in which GM1 has been added to the New York newborn screening pilot program led by Melissa Wasserstein, M.D.
    - Collaboration with Invitae, a leading genetic testing company, to support its Detect Lysosomal Storage Disorders program to encourage early diagnosis of GM1 and to provide educational information to patients and clinicians regarding clinical trials.
    - Engagement with Informed DNA to design and support a genetic screening and counselling program that will be free of charge to patients with FTD.
    - Working with investigators participating in the ALL FTD study and the Genetic Frontotemporal Dementia Initiative and supporting the Bluefield Project to Cure FTD.

Anticipated Upcoming Milestones

- Dose the first patient in the global Phase 1/2 trial for PBGM01, Imagine-1, for the treatment of infantile GM1 in the first quarter of 2021. Report initial 30-day safety and biomarker data mid-year 2021.
- Open CMC research and development site in Hopewell, NJ, in the second quarter of 2021.
- Initiate Phase 1/2 trial for PBKR03 for the treatment of early infantile Krabbe disease in the first half of 2021. Report initial 30-day safety and biomarker data in late 2021 or early 2022.
- Initiate Phase 1/2 trial for PBFT02 for the treatment of FTD-GRN in the first half of 2021. Report initial 30-day safety and biomarker data in late 2021 or early 2022.
- Continue to advance preclinical programs for PBML04 (Metachromatic leukodystrophy), PBAL05 (Amyotrophic lateral sclerosis) and PBCM06 (Charcot-Marie-Tooth Disease Type 2A), and an undisclosed adult CNS program.

Fourth Quarter and Full Year 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $304.8 million as of December 31, 2020 as compared to $158.9 million as of December 31, 2019. In January of 2021, the company raised an additional $166 million in net proceeds from a public offering.
- **Research and Development (R&D) Expenses:** R&D expenses were $27.9 million for the quarter and $81.8 million for the full year ended December 31, 2020, compared to $10.0 million and $29.7 million for the same quarter and year in 2019.
- **General and Administrative (G&A) Expenses**: G&A expenses were $10.1 million for the quarter and $30.1 million for the full year ended December 31, 2020, compared to $3.6 million and $7.0 million for the same quarter and year in 2019.
- **Net Loss**: Net loss was $38.9 million, or a net loss of $0.85 per basic and diluted share, for the quarter and $112.2 million, or a net loss of $2.91 per basic and diluted share, for the year ended December 31, 2020, compared to $13.2 million, or a net loss of $3.07 per basic and diluted share, for the quarter and $45.6 million, or a net loss of $10.77 per basic and diluted share, for the year ended December 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 6758094. 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 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)	December 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 135,002	$ 158,874
Marketable securities	169,815	—
Prepaid expenses	1,405	156
Prepaid research and development	10,961	6,745
Total current assets	317,183	165,775
Other assets	8,029	11,751
Total assets	$ 328,007	$ 178,613
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 5,265	$ 629
Accrued expenses and other current liabilities	15,910	3,052
Total current liabilities	21,175	3,681
Deferred rent	2,077	504
Other liabilities	41	76
Total liabilities	23,293	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; 45,917,084 shares issued and 45,614,807 shares outstanding at December 31, 2020 and 5,194,518 shares issued and 4,293,039 shares outstanding at December 31, 2019	4	—
Additional paid-in capital	475,617	2,410
Accumulated other comprehensive loss	(12)	—
Accumulated deficit	(170,895)	(58,663)
Total stockholders' equity (deficit)	304,714	(56,253)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 328,007	$ 178,613

Statements of Operations and Comprehensive Loss
(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
(in thousands, except share and per share data)	**2020**	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 27,932	$ 9,972	$ 81,788	$ 29,738
Acquired in-process research and development	1,000	—	1,000	500
General and administrative	10,124	3,620	30,114	6,951
Loss from operations	(39,056)	(13,592)	(112,902)	(37,189)
Change in fair value of future tranche right liability	—	—	—	(9,141)
Interest income, net	112	441	670	696
Net loss	$ (38,944)	$ (13,151)	$ (112,232)	$ (45,634)
Per share information:				
Net loss per share of common stock, basic and diluted	$ (0.85)	$ (3.07)	$ (2.91)	$ (10.77)
Weighted average common shares outstanding, basic and diluted	45,571,132	4,290,598	38,615,967	4,236,061
Comprehensive loss:				
Net loss	$ (38,944)	$ (13,151)	$ (112,232)	$ (45,634)
Unrealized loss on available-for-sale securities	28	—	(12)	—
Comprehensive loss	$ (38,916)	$ (13,151)	$ (112,244)	$ (45,634)