GBT Reports Recent Business Progress and First Quarter 2019 Financial Results

May 8, 2019

SOUTH SAN FRANCISCO, Calif., May 08, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the first quarter ended March 31, 2019.

“With a goal of getting voxelotor to the sickle cell community as quickly as possible, our efforts in the first part of 2019 have focused on preparing to submit our New Drug Application (NDA) in the second half of this year and working with the U.S. Food and Drug Administration (FDA) on the details of our post-approval confirmatory transcranial doppler (TCD) study to demonstrate voxelotor’s impact on reducing stroke risk,” said Ted W. Love, M.D., president and chief executive officer of GBT. “We also look forward to sharing 24-week efficacy data from approximately 270 patients in our Phase 3 HOPE Study at an upcoming medical meeting. This 24-week efficacy data from the full patient cohort is what will be included in our NDA submission.”

Recent Business Progress

Voxelotor

- Completed the pre-NDA meeting with the FDA and announced the agency’s agreement to a rolling submission for voxelotor for the potential treatment of sickle cell disease (SCD).
- Results from the company’s Phase 1/2 study demonstrating the safety, tolerability, pharmacokinetic and pharmacodynamic properties of voxelotor in patients with SCD were published in *Blood*.

Corporate

- Launched the Access to Excellent Care for Sickle Cell Patients Pilot Program (ACCEL) to provide grant funding to support novel projects aimed at improving access to high-quality healthcare for individuals with SCD in the United States.
- Expanded the management team with the appointment of Brian Cathers, Ph.D., as chief scientific officer. Dr. Cathers is an accomplished leader with nearly 20 years of research and drug development experience in the biopharmaceutical industry, including most recently serving as executive director of the Protein Homeostasis Thematic Center of Excellence at Celgene Corporation.

Financial Results for the Three Months Ended March 31, 2019
Cash, cash equivalents and marketable securities totaled $575.1 million at March 31, 2019, compared with $591.8 million at December 31, 2018.

Net loss for the three months ended March 31, 2019, was $48.9 million compared with $41.6 million for the same period in 2018. Basic and diluted net loss per share for the three months ended March 31, 2019, was $0.87 compared with $0.87 for the same period in 2018. We expect our net loss to increase during 2019 as we expand our manufacturing efforts for voxelotor, commence the conduct of additional clinical studies of voxelotor in SCD, continue our existing SCD clinical trials and increase general and administrative (G&A) spending as we buildout our commercial infrastructure and prepare for the potential commercial launch of voxelotor in SCD.

Research and development (R&D) expenses for the three months ended March 31, 2019, were $34.5 million compared with $29.9 million for the same period in 2018. The increase in R&D expenses is primarily attributable to increased employee-related costs, including non-cash stock compensation, and increased consulting spend associated with NDA filing activities. Total R&D stock-based compensation expense incurred for the three months ended March 31, 2019, was $4.0 million, compared with $3.0 million for the same period in 2018.

General and administrative (G&A) expenses for the three months ended March 31, 2019, were $18.1 million compared with $12.8 million for the same period in 2018. The increase in G&A expenses is primarily attributable to increased employee-related costs, including non-cash stock compensation, and increased level of spend associated with the buildout of our commercial infrastructure. Total G&A stock-based compensation expense incurred in the three months ended March 31, 2019, was $5.4 million, compared with $4.8 million for the same period in 2018.

About GBT
GBT is a clinical-stage biopharmaceutical company determined to discover, develop and deliver innovative treatments that provide hope to underserved patient communities. GBT is developing two therapies for the potential treatment of sickle cell disease, including its late-stage product candidate, voxelotor, as an oral, once-daily therapy. To learn more, please visit www.gbt.com and follow the company on Twitter @GBT_news.

Forward-Looking Statements
Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995, including statements about GBT’s anticipated public offering, anticipated use of proceeds and other statements containing the words “anticipate,” “planned,”

"believe," "forecast," "estimated," "expected," and "intend," among others. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements we make in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. We intend these forward-looking statements, including statements regarding our plan to submit a rolling NDA for voxelotor under an accelerated regulatory approval pathway, the availability of, and sufficiency of our data to support, accelerated regulatory approval, the therapeutic potential and safety profile of voxelotor, including the potential to be a disease-modify therapy for SCD, our plan to initiate a TCD confirmatory study, our potential commercial launch, our ability to implement and complete our clinical development plans for voxelotor, our ability to engage in continued discussions with the FDA and the outcome of our discussions with the FDA, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including data from patients enrolled in our Phase 3 HOPE Study, including the 24-week efficacy data from approximately 270 patients, and data in our ongoing Phase 2a HOPE-KIDS 1 Study), our plan to include this 24-week efficacy data in our NDA, regulatory review and actions relating to voxelotor, and the timing of these events, to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. We can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved, and furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, the risks that our clinical and preclinical development activities may be delayed or terminated for a variety of reasons, that results of clinical trials may be subject to differing interpretations, that regulatory authorities may disagree with our clinical development plans or require additional studies or data to support further clinical investigation of our product candidates, that drug-related adverse events may be observed in clinical development, and that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2019, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Operating expenses:		
Research and development	$ 34,468	$ 29,944
General and administrative	18,055	12,751
Total operating expenses	52,523	42,695
Loss from operations	(52,523)	(42,695)
Other income (expense):		
Interest income, net	3,650	1,173
Other expenses, net	(50)	(34)
Total other income, net	3,600	1,139
Net loss	$ (48,923)	$ (41,556)
Basic and diluted net loss per common share	$ (0.87)	$ (0.87)
Weighted-average number of shares used in computing basic and diluted net loss per common share	56,231,587	47,770,023

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(In thousands)

	March 31, 2019 (Unaudited)	**December 31, 2018**
Assets		
Current assets:		
Cash and cash equivalents	$ 156,836	$ 275,357
Short-term marketable securities	300,431	202,177
Prepaid expenses and other current assets	8,222	8,246
Total current assets	465,489	485,780
Property and equipment, net	13,090	14,981
Long-term marketable securities	117,811	114,281
Operating lease right-of-use assets	14,075	—
Other assets	2,595	2,601
Total assets	$ 613,060	$ 617,643

Liabilities and Stockholders' Equity		
Current liabilities	$ 32,091	$ 33,773
Operating lease liabilities, noncurrent	24,401	—
Other liabilities, noncurrent	30	11,071
Total liabilities	56,522	44,844
Total stockholders' equity	556,538	572,799
Total liabilities and stockholders' equity	$ 613,060	$ 617,643

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Source: Global Blood Therapeutics, Inc.