GBT Reports Recent Business Progress and Fourth Quarter and Year-End 2018 Financial Results

February 27, 2019

Pre-NDA Meeting Completed and Company Announces FDA Agreed to a Rolling Submission of New Drug Application for Voxelotor for the Potential Treatment of Sickle Cell Disease

SOUTH SAN FRANCISCO, Calif., Feb. 27, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the fourth quarter and year ended December 31, 2018.

“Last year was successful for GBT as we achieved several important clinical and regulatory milestones with voxelotor for the potential treatment of sickle cell disease (SCD). Importantly, the U.S. Food and Drug Administration (FDA) agreed with our proposal to use an accelerated approval pathway for voxelotor using hemoglobin as the primary endpoint, and that transcranial doppler (TCD) flow velocity would be an acceptable primary endpoint in a post-approval confirmatory study to demonstrate stroke risk reduction,” said Ted W. Love, M.D., president and chief executive officer of GBT. “Our momentum in 2019 continues to be driven by our passion to make voxelotor, a potentially disease-modifying therapy, available to the sickle cell community. I am pleased to share that the FDA has agreed to a rolling submission of our New Drug Application for voxelotor. Additionally, we look forward to initiating our TCD post-approval confirmatory study later this year. Our preparations towards becoming a commercial stage organization in the first half of 2020 are also well underway.”

Recent Business Progress

Sickle Cell Disease

- Announced that the FDA agreed with the company’s proposal to use an accelerated approval pathway for voxelotor for the treatment of SCD with hemoglobin as the primary endpoint, and that TCD would be an acceptable primary endpoint in a post-approval confirmatory study to demonstrate stroke risk reduction.
- Presented results from four studies at the 60th American Society of Hematology Annual Meeting and Exposition:
  - Twenty-four-week efficacy and safety results from the Phase 3 HOPE Study of voxelotor in 154 patients age 12 and older with SCD that demonstrated rapid, robust and sustained improvements in hemoglobin levels and measures of hemolysis with a favorable safety and tolerability profile;
  - An interim analysis of data from the 1500 mg cohort of the HOPE-KIDS 1 Study that further supported voxelotor’s favorable safety and tolerability profile in pediatric patients;
  - A systemic literature review and meta-analysis that showed a statistically significant relationship between chronic anemia and negative clinical outcomes like risk of stroke and mortality in SCD patients, and that an increase in hemoglobin of 1 g/dL or greater might reduce the risk of stroke and mortality by 41 percent and 64 percent, respectively; and
  - A meta-analysis of data on the societal costs of SCD in the United States that found that people with SCD are estimated to live two decades less than those without SCD, and this reduced life expectancy translates into approximately $700,000 in lost lifetime earnings.

Corporate

- Raised approximately $162.1 million in net proceeds, after deducting underwriting costs and commissions and estimated offering expenses, from an underwritten public offering in December 2018 and related exercise of the over-allotment option in January 2019.
- Appointed Dawn Svoronos to the company’s board of directors. Ms. Svoronos has more than 30 years of experience in the biopharmaceutical industry in the United States, Canada, Europe and Asia, including a nearly 25-year tenure at Merck & Co., Inc.

Financial Results for the Fourth Quarter and Year-End 2018

Cash, cash equivalents and marketable securities totaled $591.8 million at December 31, 2018, compared with $329.4 million at December 31, 2017. This amount excludes approximately $21.2 million that resulted from the underwriters exercising their over-allotment option in January 2019.

Net loss for the three months ended December 31, 2018, was $49.2 million compared with $41.3 million for the same period in 2017. Basic and diluted net loss per share for the three months ended December 31, 2018, was $0.93 compared with $0.95 for the same period in 2017. Net loss for the year

ended December 31, 2018, was $174.2 million compared with $117.0 million for the same period in 2017. Basic and diluted net loss per share for the year ended December 31, 2018, was $3.41 compared with $2.76 for the same period in 2017. We expect our net loss to increase during 2019 as we expand our manufacturing efforts for voxelotor, conduct additional clinical studies supporting voxelotor, 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 December 31, 2018, were $36.8 million compared with $31.3 million for the same period in 2017. R&D expenses for the year ended December 31, 2018, were $131.3 million compared with $87.8 million for the same period in 2017. The increase in R&D expenses for both comparative periods is primarily attributable to increased expenses for the Phase 2a HOPE-KIDS 1 Study and the pivotal HOPE Study as well as higher levels of manufacturing activities to support the clinical programs and prepare for commercial launch. Total R&D non-cash stock compensation expense incurred for the three months ended December 31, 2018, was $3.2 million, compared with $1.8 million for the same period in 2017. Total R&D non-cash stock compensation expense incurred for the year ended December 31, 2018, was $12.7 million, compared with $5.9 million for the same period in 2017.

G&A expenses for the three months ended December 31, 2018, were $15.3 million compared with $10.6 million for the same period in 2017. G&A expenses for the year ended December 31, 2018, were $51.4 million compared with $31.4 million for the same period in 2017. The increase in G&A expenses for both comparative periods is primarily attributable to increased employee-related costs, including non-cash stock compensation, and increased professional and consulting services associated with the growth of the company's operations. Total G&A non-cash stock compensation expense incurred in the three months ended December 31, 2018, was $4.4 million, compared with $2.9 million for the same period in 2017. Total G&A non-cash stock compensation expense incurred in the year ended December 31, 2018, was $17.3 million, compared with $7.8 million for the same period in 2017.

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 its lead product candidate, voxelotor, as an oral, once-daily therapy for sickle cell disease. 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 an NDA for voxelotor under an accelerated regulatory approval pathway, the availability of a rolling submission of our NDA, 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, the potential for TCD flow velocity to serve as an acceptable primary endpoint in a confirmatory study, our 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, the potential for an increase in hemoglobin of 1 g/dL or greater to reduce the risk of stroke and mortality in patients with SCD, 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, and data in our ongoing Phase 2a HOPE-KIDS 1 Study), 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 September 30, 2018, 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

(In thousands, except share and per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Operating expenses:	(Unaudited)	(Unaudited)		
Research and development	$ 36,765	$ 31,295	$ 131,307	$ 87,807
General and administrative	15,319	10,620	51,435	31,438
Total operating expenses	52,084	41,915	182,742	119,245
Loss from operations	(52,084)	(41,915)	(182,742)	(119,245)
Other income (expense):				

Interest income, net	2,850	699	8,618	2,555
Other income (expenses), net	33	(36)	(69)	(334)
Total other income, net	2,883	663	8,549	2,221
Net loss	$ (49,201)	$ (41,252)	$ (174,193)	$ (117,024)
Basic and diluted net loss per common share	$ (0.93)	$ (0.95)	$ (3.41)	$ (2.76)
Weighted-average number of shares used in computing basic and diluted net loss per common share	52,972,225	43,810,504	51,150,728	42,323,686

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(In thousands)

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 275,357	$ 198,332
Short-term marketable securities	202,177	116,493
Prepaid expenses and other current assets	8,246	9,487
Total current assets	485,780	324,312
Property and equipment, net	14,981	16,571
Long-term marketable securities	114,281	14,607
Other assets	2,601	1,230
Total assets	$ 617,643	$ 356,720
Liabilities and Stockholders' Equity		
Current liabilities	$ 33,773	$ 26,264
Other liabilities, noncurrent	11,071	11,652
Total liabilities	44,844	37,916
Total stockholders' equity	572,799	318,804
Total liabilities and stockholders' equity	$ 617,643	$ 356,720

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Global Blood Therapeutics, Inc.