GBT Reports Recent Business Progress and Fourth Quarter and Year-End 2017 Financial Results

February 27, 2018

SOUTH SAN FRANCISCO, Calif., Feb. 27, 2018 (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, 2017.

"We continue to build positive momentum toward bringing voxelotor, the first and only sickle cell disease (SCD) treatment to receive Breakthrough Therapy Designation from the U.S. Food and Drug Administration, to the SCD community," said Ted W. Love, M.D., president and chief executive officer of GBT. "We expect to continue this momentum in 2018, as we expect to announce top-line clinical data from our ongoing voxelotor clinical programs - HOPE Study Part A and HOPE-KIDS 1 Study. We are also working diligently to diversify our pipeline, both through our internal research as well as through business development efforts, by exploring innovative opportunities that have the potential to make a significant difference in the lives of underserved patient communities."

Recent Business Progress

Sickle Cell Disease (SCD)

- Received acceptance of four abstracts for poster presentations at the upcoming 2018 American Society of Pediatric Hematology / Oncology (ASPHO) Conference, which will be held May 2-5 in Pittsburgh.
- Received Breakthrough Therapy Designation from the U.S. Food and Drug Administration for voxelotor for the treatment of SCD in January 2018.
- Presented results from six studies at the 59th American Society of Hematology Annual Meeting & Exposition, including results from the ongoing Phase 2a HOPE-KIDS 1 Study, which demonstrated that voxelotor provided clinical benefits in children age 12 to 17, and a case series of seven patients with severe SCD who were treated with voxelotor through single-patient compassionate access.
- Announced that the independent Data and Safety Monitoring Board (DSMB) for the Phase 3 HOPE (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) Study evaluating voxelotor for the treatment of SCD, completed its first scheduled clinical safety review. Based on the available safety data to date, the DSMB recommended that this pivotal study continue as planned and approved initiation of enrollment in adolescent patients age 12 to 17.

Corporate

- Raised approximately $110.9 million in net proceeds, after deducting underwriting costs and commissions and estimated offering expenses, from an underwritten public offering in December 2017 and related exercise of the over-allotment option in January 2018.
- Appointed Wendy L. Yarno to the Company's board of directors. Ms. Yarno has more than 25 years of experience in the biopharmaceutical industry, including serving as chief marketing officer for Merck & Co. Inc.

Financial Results for the Fourth Quarter and Year-End 2017
Cash, cash equivalents and marketable securities totaled $329.4 million at December 31, 2017, compared with $197.3 million at December 31, 2016. This amount excludes approximately $14.5 million that resulted from the underwriters exercising their over-allotment option in January 2018.

Net loss for the three months ended December 31, 2017, was $41.3 million compared with $27.2 million for the same period in 2016. Basic and diluted net loss per share for the three months ended December 31, 2017, was $0.94 compared with $0.74 for the same period in 2016. Net loss for the year ended December 31, 2017, was $117.0 million compared with $82.5 million for the same period in 2016. Basic and diluted net loss per share for the year ended December 31, 2017, was $2.76 compared with $2.48 for the same period in 2016. We expect our net loss to increase during 2018 as we expand our manufacturing efforts for voxelotor, increase patient enrollment in our various SCD clinical trials and increase general and administrative (G&A) spending as we prepare for the potential commercial launch of voxelotor in SCD.

Research and development (R&D) expenses for the three months ended December 31, 2017, were $31.3 million compared with $20.9 million for the same period in 2016. R&D expenses for the year ended December 31, 2017, were $87.8 million compared with $62.2 million for the same period in 2016. 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. Total R&D stock compensation expense incurred for the three months ended December 31, 2017, was $1.8 million, compared with $1.9 million for the same period in 2016. Total R&D stock compensation expense incurred for the year ended December 31,

2017, was $5.9 million, compared with $4.2 million for the same period in 2016.

G&A expenses for the three months ended December 31, 2017, were $10.6 million compared with $6.6 million for the same period in 2016. G&A expenses for the year ended December 31, 2017, were $31.4 million compared with $21.0 million for the same period in 2016. The increase in G&A expenses for both comparative periods is primarily attributable to increased employee-related costs and increased professional and consulting services associated with the growth of the Company's operations. Total G&A stock compensation expense incurred in the three months ended December 31, 2017, was $2.9 million, compared with $1.5 million for the same period in 2016. Total G&A stock compensation expense incurred in the year ended December 31, 2017, was $7.8 million, compared with $5.1 million for the same period in 2016.

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

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 the therapeutic potential and safety profile of voxelotor (previously called GBT440), our ability to implement and complete our clinical development plans for voxelotor, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including our ongoing Phase 3 HOPE Study and our ongoing Phase 2a HOPE-KIDS 1 Study), our ability to diversify our pipeline, 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, 2017, 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,	
	2017	2016	2017	2016
Operating expenses:	(Unaudited)	(Unaudited)		
Research and development	$ 31,295	$ 20,908	$ 87,807	$ 62,163
General and administrative	10,620	6,552	31,438	20,964
Total operating expenses	41,915	27,460	119,245	83,127
Loss from operations	(41,915)	(27,460)	(119,245)	(83,127)
Other income (expense):				
Interest income, net	698	252	2,555	659
Other expenses, net	(36)	—	(334)	—
Total other income, net	662	252	2,221	659
Net loss	$ (41,253)	$ (27,208)	$ (117,024)	$ (82,468)
Basic and diluted net loss per common share	$ (0.94)	$ (0.74)	$ (2.76)	$ (2.48)
Weighted-average number of shares used in computing basic and diluted net loss per common share	43,810,504	36,580,582	42,323,686	33,207,382

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(In thousands)

	December 31, 2017	December 31, 2016 (1)
Assets		
Current assets:		

Cash and cash equivalents	$ 198,332	$ 92,072
Short-term marketable securities	116,493	55,202
Prepaid expenses and other current assets	9,487	2,495
Total current assets	324,312	149,769
Long-term marketable securities	14,607	50,058
Other assets	17,801	2,560
Total assets	$ 356,720	$ 202,387
Liabilities and Stockholders' Equity		
Current liabilities	$ 26,264	$ 15,515
Other liabilities, noncurrent	11,652	563
Total liabilities	37,916	16,078
Total stockholders' equity	318,804	186,309
Total liabilities and stockholders' equity	$ 356,720	$ 202,387

(1) Derived from the audited financial statements, included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 filed with the Securities and Exchange Commission on March 13, 2017.

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