GBT Reports Recent Business Progress and Provides Second Quarter 2018 Financial Results

August 2, 2018

SOUTH SAN FRANCISCO, Calif., Aug. 02, 2018 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported business progress and financial results for the second quarter ended June 30, 2018.

"We continue to advance our voxelotor clinical development program with the goal of bringing this potentially transformative treatment to the sickle cell disease, or SCD, community as soon as possible," said Ted W. Love, M.D., president and chief executive officer of GBT. "As part of that effort, we are pleased that the top-line data from Part A of our Phase 3 HOPE Study of voxelotor demonstrated improvement in hemolytic anemia as assessed by the statistically significant increase in hemoglobin. Based on the well-established association between chronic hemolytic anemia and SCD-related morbidity and mortality, we have always believed that hemoglobin is an important measure of clinical benefit and that is why we chose it as the primary endpoint in the HOPE Study. Given these encouraging data and the critical need for new treatments in SCD, we believe voxelotor meets the standard for an accelerated approval. We have initiated discussions with the U.S. Food and Drug Administration based on all available efficacy and safety data from the voxelotor clinical development program, and we look forward to providing further updates in the near future."

Recent Business Progress

Voxelotor

- Announced positive top-line data from Part A of the Phase 3 HOPE (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) Study, which is evaluating voxelotor for the treatment of SCD in patients age 12 and older.
    - GBT intends to present data from Part A of the Phase 3 HOPE Study at a major medical meeting later this year.
- Presented four encore presentations related to the voxelotor SCD program during the Foundation for Sickle Cell Disease Research's (FSCDR) 12th Annual SCD Research and Educational Symposium.
- Presented new 24-week data in patients treated with the 900 mg dose of voxelotor from the Phase 2a HOPE-KIDS 1 Study in adolescents ages 6 to 17 years with SCD at the 23rd European Hematology Association (EHA) Congress.
    - GBT intends to present data from the 1500 mg cohort from its HOPE-KIDS 1 Study at a major medical meeting later this year.
- *Drug Design, Development and Therapy* published results of a study showing that a cost-effective, simple, rapid and highly adaptable oxygen dissociation assay developed by GBT allows the rapid characterization of hemoglobin-oxygen affinity modifiers.
- *American Journal of Hematology* published results from a case series of patients with severe SCD treated with voxelotor through compassionate access.
- *Hematology Reports* published results from two separate studies:
    - A case study of a patient with SCD and severe chronic jaundice demonstrating that voxelotor reduced bilirubin levels and improved clinical manifestations of jaundice.
    - An in vitro study demonstrating that voxelotor increased hemoglobin-oxygen affinity and reversed in vitro sickling of previously sickled red blood cells under hypoxic conditions. These results suggest that voxelotor, besides preventing sickling of red blood cells, may mitigate vaso-occlusion and microvascular dysfunction by reversing sickling of circulating sickled red blood cells in vivo.

Financial Results for the Three Months Ended June 30, 2018

Cash, cash equivalents and marketable securities totaled $514.0 million at June 30, 2018, compared with $329.4 million at December 31, 2017.

Net loss for the three months ended June 30, 2018, was $40.4 million compared with $23.9 million for the same period in 2017. Basic and diluted net loss per share for the three months ended June 30, 2018, was $0.78 compared with $0.55 for the same period in 2017.

Research and development (R&D) expenses for the three months ended June 30, 2018, were $31.6 million compared with $18.3 million for the same period in 2017. The increase in R&D expenses is primarily attributable to increased expenses for the Phase 2a HOPE-KIDS 1 Study and the Phase 3 HOPE Study. Total R&D non-cash stock compensation expense incurred for the three months ended June 30, 2018, was $3.8 million compared with $1.4 million for the same period in 2017.

General and administrative (G&A) expenses for the three months ended June 30, 2018, were $10.9 million compared with $6.2 million for the same

period in 2017. The increase in G&A expenses is primarily attributable to increased employee-related costs, including non-cash stock compensation expense, 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 June 30, 2018, was $4.0 million, compared with $1.2 million for the same period in 2017.

About Global Blood Therapeutics

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, 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 those discussions, the availability of accelerated approval, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including our ability to generate additional data from patients enrolled in our ongoing Phase 3 HOPE Study), the sufficiency of our data to support an application for regulatory approval, 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, including the sufficiency of our clinical data and of our primary and other key endpoints in our Phase 3 HOPE Study of voxelotor to support approval, or require additional studies or data to support approval or further clinical investigation of voxelotor, that drug-related adverse events may be observed in clinical development, that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, and that we may need to devote additional time and resources to meet these regulatory requirements, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017, and our Quarterly Report on Form 10-Q for the quarter ended June 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
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	$ 31,573	$ 18,278	$ 61,517	$ 35,561
General and administrative	10,914	6,152	23,665	12,590
Total operating expenses	42,487	24,430	85,182	48,151
Loss from operations	(42,487)	(24,430)	(85,182)	(48,151)
Other income (expense):				
Interest income, net	2,115	689	3,287	1,130
Other income (expenses), net	4	(142)	(29)	(194)
Total other income, net	2,119	547	3,258	936
Net loss	$ (40,368)	$ (23,883)	$ (81,924)	$ (47,215)
Basic and diluted net loss per common share	$ (0.78)	$ (0.55)	$ (1.65)	$ (1.15)
Weighted-average number of shares used in computing basic and diluted net loss per common share	51,742,904	43,063,996	49,767,633	41,112,266

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets
(In thousands)

	June 30, 2018 (Unaudited)	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 342,103	$ 198,332
Short-term marketable securities	120,381	116,493
Prepaid expenses and other current assets	7,051	9,487
Total current assets	469,535	324,312

Property and equipment, net	17,097	16,571
Long-term marketable securities	51,487	14,607
Other assets	1,119	1,230
Total assets	$ 539,238	$ 356,720
Liabilities and Stockholders' Equity		
Current liabilities	$ 21,287	$ 26,264
Other liabilities, noncurrent	11,398	11,652
Total liabilities	32,685	37,916
Total stockholders' equity	506,553	318,804
Total liabilities and stockholders' equity	$ 539,238	$ 356,720

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