Global Blood Therapeutics Reports Recent Business Progress and Provides First Quarter 2016 Financial Results

May 12, 2016

SOUTH SAN FRANCISCO, Calif., May 12, 2016 /PRNewswire/ -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT), a biopharmaceutical company developing novel therapeutics for the treatment of grievous blood-based disorders with significant unmet needs, today reported business progress and financial results for the first quarter ended March 31, 2016.

"We began 2016 by expanding our management team and continuing to build clinical evidence for our lead program, GBT440 for the treatment of sickle cell disease (SCD)," said Ted W. Love, M.D., chief executive officer of GBT. "As we look ahead to the upcoming European Hematology Association (EHA) Annual Congress, we plan to share longer-term data for GBT440. We expect this data will continue to demonstrate, at anticipated therapeutic doses, that GBT440 has a durable, positive impact across hemolysis in SCD patients and continues to be well tolerated, further validating what we've seen so far. The proof-of-concept data we continue to obtain will inform the design of the pivotal program that we expect to initiate in the second half of 2016."

Recent Business Progress

Corporate

- Appointed Jeffrey Farrow as chief financial officer. Mr. Farrow has been a leader in finance, risk management and investor relations for several research, development and commercial-stage public biotech companies, including Hyperion Therapeutics, Inc. and ZS Pharma, Inc.

Sickle Cell Disease

- Received acceptance of two poster presentations at the upcoming EHA Annual Congress, which will be held on June 9-12 in Copenhagen, Denmark. The presentations will take place on Friday, June 10, and will include additional data from the ongoing Phase 1/2 GBT440-001 study including 28-day results from three dosing cohorts of GBT440, 90-day data from a cohort of patients taking 700 mg of GBT440, and additional data on the pharmacokinetics and pharmacodynamics of GBT440. Abstracts will be posted online at www.ehaweb.org on May 19, with the full GBT440 data update available during the presentations.
- Presented two oral presentations at the Sickle Cell Disease Research and Educational Symposium on April 17 in Fort Lauderdale, Fla. These presentations included data previously presented at the 2015 American Society of Hematology (ASH) Annual Meeting and an update on a new tool to measure patient-reported outcomes in patients with SCD.

Hypoxemic Pulmonary Disorders

- Received acceptance of a poster presentation at the upcoming American Thoracic Society International Conference, which will be held on May 13-18 in San Francisco, CA. The presentation describes the effects of an analog of GBT440 in a mouse model of hypoxic acute lung injury.
    - **Poster:** A Drug That Increases Oxygen Affinity of Hemoglobin, Improved Survival During Murine Hypoxic Acute Lung Injury
      Presenter: N.D. Putz, Bachelor of Arts
      Session: Respiratory Failure: Mechanistic Insights from Lung Injury Models
      Date: Tuesday, May 17, 2016
      Time: 2:15 p.m. – 4:15 p.m. (PT)

Financial Results for the Three Months Ended March 31, 2016

Cash and cash equivalents totaled $134.0 million at March 31, 2016 compared with $148.5 million at December 31, 2015.

Net loss and comprehensive loss for the three months ended March 31, 2016 was $16.6 million compared with $7.4 million for the same period in 2015. Basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2016 was $0.56 compared with

$4.22 for the same period in 2015.

Research and development (R&D) expenses for the three months ended March 31, 2016 were $12.4 million compared with $6.1 million for the same period in 2015. The increase in R&D expenses is primarily attributable to increased expenses related to the Company's development of GBT440 for the treatment of SCD and to the ongoing pre-clinical studies for the treatment of hypoxemic pulmonary disorders and hereditary angioedema.

General and administrative (G&A) expenses for the three months ended March 31, 2016 were $4.3 million compared with $1.3 million for the same period in 2015. The increase in G&A expenses is primarily attributable to increased employee-related costs associated with the hiring of additional business personnel and additional professional and consulting services due to our transition from a private to a public company.

About Global Blood Therapeutics

Global Blood Therapeutics, Inc. (GBT) is a clinical-stage biopharmaceutical company dedicated to discovering, developing, and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need. GBT is developing its initial product candidate, GBT440, as an oral, anti-polymerization therapy for sickle cell disease (SCD) and is currently evaluating GBT440 in SCD patients in a randomized, placebo-controlled, double-blind Phase 1/2 clinical trial. In addition to GBT440 for the treatment of SCD, GBT is engaged in research and development activities targeted toward hypoxemic pulmonary disorders, including idiopathic pulmonary fibrosis (IPF) and other lung disorders, as well as hereditary angioedema (HAE). To learn more, please visit: www.globalbloodtx.com.

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 GBT440, our ability to receive data from our ongoing Phase 1/2 clinical study of GBT440, and our ability to initiate our pivotal program for GBT440 in SCD, 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 regulatory authorities may disagree with our clinical development plans or require additional studies or data to support further clinical investigation of our product candidate, and that drug-related adverse events may be observed in later stages of clinical development, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, 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 Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

	(Unaudited) Three Months Ended March 31,	
	2016	**2015**
Operating expenses:		
Research and development	$ 12,415	$ 6,069
General and administrative	4,302	1,298
Related party expenses	-	53
Total operating expenses	16,717	7,420
Loss from operations	(16,717)	(7,420)
Interest income	117	3
Net loss and comprehensive loss	$ (16,600)	$ (7,417)
Net loss attributable to common stockholders	$ (16,600)	$ (8,657)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.56)	$ (4.22)
Weighted-average number of shares used in computing net loss per share attributable to common stockholders, basic and diluted	29,441,404	2,052,874

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Balance Sheets

(In thousands, except share and per share amounts)

March 31, 2016	**December 31, 2015**
(Unaudited)	

Assets		
Current assets:		
Cash and cash equivalents	$ 133,984	$ 148,502
Prepaid expenses and other current assets	2,554	2,318
Total current assets	136,538	150,820
Other assets	2,433	2,254
Total assets	$ 138,971	$ 153,074
Liabilities and Stockholders' Equity		
Current liabilities	$ 11,495	$ 10,723
Other noncurrent liabilities	1,354	1,556
Total liabilities	12,849	12,279
Total stockholders' equity	126,122	140,795
Total liabilities and stockholders' equity	$ 138,971	$ 153,074

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/global-blood-therapeutics-reports-recent-business-progress-and-provides-first-quarter-2016-financial-results-300267879.html

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