Global Blood Therapeutics Reports Recent Business Progress and Provides Fourth Quarter and Year-End 2015 Financial Results

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SOUTH SAN FRANCISCO, Calif., March 29, 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 fourth quarter and year ended December 31, 2015.

"In 2015, we successfully transformed GBT into a public company, while demonstrating clinical proof of concept for our lead program, GBT440, for the treatment of sickle cell disease," said Ted W. Love, M.D., chief executive officer of GBT. "2016 will be focused on building on this momentum with the execution of several key milestones, including driving toward the initiation of a pivotal program with GBT440 in adults with sickle cell disease and generating results from a Phase 2a proof of concept study of GBT440 in patients with idiopathic pulmonary fibrosis."

Recent Business Progress

Corporate

- Appointed three new members to the Company's Board of Directors, including Scott Morrison, a financial expert who was previously Ernst & Young's U.S. Life Sciences Leader, Mike Bonney, previously CEO of Cubist Pharmaceuticals and a partner at Third Rock Ventures, and Glenn Pierce, M.D., Ph.D., a hematology R&D expert retired from Biogen. The Company also announced that Kevin Starr, a partner at Third Rock Ventures, retired from the Board.

Sickle Cell Disease (SCD)

- Presented positive clinical data at the 2015 American Society of Hematology (ASH) Annual Meeting and Exposition from the ongoing Phase 1/2 clinical trial (the GBT440-001 study) supporting the potential of GBT440, an oral, anti-polymerization drug candidate that has the potential to be a disease-modifying treatment for SCD. Specifically, the data showed that GBT440 has the potential to inhibit polymerization of sickle hemoglobin, reduce red blood cell hemolysis, reduce anemia, improve tissue oxygen delivery, reduce the number of sickled red blood cells, and reduce inflammation. The results included data from 30 patients with SCD who have completed 28 days of treatment on GBT440 or placebo, and reinforce and expand on the safety and efficacy data previously reported in an initial cohort of eight patients.
- Received orphan drug designation for GBT440 for the treatment of SCD from the U.S. Food and Drug Administration (FDA).
- Initiated Part C of the GBT440-001 study to evaluate the effect of 90 days of treatment with GBT440 or placebo.
- Received acceptance of two abstracts for presentation at the upcoming Sickle Cell Disease Research and Educational Symposium taking place April 14 –April 18 in Fort Lauderdale, Fla. The data will be encore presentations of the ASH data.

2016 Anticipated Milestones

Sickle Cell Disease

- Present 90-day data in a cohort of patients with SCD from Part C of the ongoing Phase 1/2 study (GBT440-001).
- Present data from the completion of 28 day cohorts, including 1,000 mg of GBT440, in patients with SCD from Part B of the ongoing Phase 1/2 study.
- Initiate a Phase 2 pharmacokinetics (PK) study of GBT440 in pediatric SCD patients.
- Initiate a pivotal program of GBT440 in adults, subject to agreement with regulatory authorities.
- Explore the effect of GBT440 in SCD patients with HbSC and HbSbeta+ thalassemia genotypes.

Hypoxemic Pulmonary Disorders

- Initiate a Phase 2a proof of concept study evaluating the effects of GBT440 on oxygen saturation in patients with idiopathic pulmonary fibrosis (IPF).

- Initiate a Phase 2b IPF study, informed by the Phase 2a study results.
- Initiate a clinical study in an acute hypoxemic pulmonary disorder, informed by the Phase 2a IPF study results.

Hereditary Angioedema

- Complete IND-enabling toxicology studies, submit an IND, and initiate a Phase 1 study for GBT18713, an orally bioavailable kallikrein inhibitor.

Fourth Quarter and Year-End Financial Results

Cash and cash equivalents totaled $148.5 million at December 31, 2015 compared with $52.1 million at December 31, 2014, reflecting the $126.2 million of net proceeds from our initial public offering in August 2015.

Net loss and comprehensive loss for the three months ended December 31, 2015 was $15.6 million compared with $5.3 million for the same period in 2014. Net loss and comprehensive loss for the year ended December 31, 2015 was $46.4 million compared with $20.8 million for the same period in 2014. Basic and diluted net loss per share attributable to common stockholders for the three months ended December 31, 2015 was $0.53 compared with $3.27 for the same period in 2014. Basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2015 was $3.95 compared with $14.20 for the same period in 2014.

Research and development (R&D) expenses for the three months ended December 31, 2015 were $11.4 million compared with $4.1 million for the same period in 2014. R&D expenses for the year ended December 31, 2015 were $36.7 million compared with $16.3 million for the same period in 2014. The increase in R&D expenses for both comparative periods is primarily attributable to increased expenses related to the Company's development of GBT440 for the treatment of SCD, including the ongoing Phase 1/2 clinical trial, and to the licensing of related intellectual property.

General and administrative (G&A) expenses for the three months ended December 31, 2015 were $4.2 million compared with $1.3 million for the same period in 2014. G&A expenses for the year ended December 31, 2015 were $9.7 million compared with $4.2 million for the same period in 2014. The increase in G&A expenses for both comparative periods is primarily attributable to higher employee-related costs associated with the growth of the Company's operations and additional professional and consulting services related to being 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 which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act. We intend these forward-looking statements, including statements regarding the therapeutic potential of GBT440 and our ability to receive data from our ongoing Phase 1/2 clinical study of GBT440, initiate our planned Phase 2 PK study of GBT440 in pediatric SCD patients, gain agreement from regulatory authorities on our pivotal program for GBT440 in adults, initiate our pivotal program, initiate our planned Phase 2a and Phase 2b clinical studies of GBT440 in IPF, successfully complete IND-enabling studies, submit an IND and commence a Phase 1 study of GBT18713 in hereditary angioedema, 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 the prospectus for our initial public offering of common stock that was filed with the U.S. Securities and Exchange Commission (the "SEC") on August 12, 2015, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the SEC. 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 December 31,		Year Ended December 31,	
	2015	2014	2015	2014
Operating expenses:				
Research and development	$ 11,400	$ 4,063	$ 36,657	$ 16,324
General and administrative	4,198	1,251	9,736	4,187
Total operating expenses	15,598	5,314	46,393	20,511

Loss from operations	(15,598)	(5,314)	(46,393)	(20,511)
Change in fair value of Series A redeemable convertible preferred stock liability	—	—	—	(297)
Interest income	13	1	33	1
Net loss and comprehensive loss	$ (15,585)	$ (5,313)	$ (46,360)	$ (20,807)
Net loss attributable to common stockholders	$ (15,585)	$ (6,214)	$ (50,540)	$ (23,772)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.53)	$ (3.27)	$ (3.95)	$ (14.20)
Weighted-average number of shares used in computing net loss per share attributable to common stockholders, basic and diluted	29,317,707	1,901,322	12,806,697	1,673,919

GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Balance Sheets
(In thousands, except share and per share amounts)

	December 31,	
	2015	**2014**
Assets		
Current assets:		
Cash and cash equivalents	$ 148,502	$ 52,069
Prepaid expenses and other current assets	2,318	1,524
Total current assets	150,820	53,593
Other assets	2,254	2,163
Total assets	$ 153,074	$ 55,756
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities	$ 10,723	$ 2,537
Other noncurrent liabilities	1,556	384
Total liabilities	12,279	2,921
Redeemable convertible preferred stock	—	102,161
Total stockholders' equity (deficit)	140,795	(49,326)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 153,074	$ 55,756

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/global-blood-therapeutics-reports-recent-business-progress-and-provides-fourth-quarter-and-year-end-2015-financial-results-300242338.html

SOURCE Global Blood Therapeutics, Inc.

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