



GBT Reports Recent Business Progress and Second Quarter 2019 Financial Results

August 7, 2019

SOUTH SAN FRANCISCO, Calif., Aug. 07, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the second quarter ended June 30, 2019.

"We accomplished several significant milestones in the second quarter of this year, including the simultaneous presentation and publication of additional data from the HOPE Study demonstrating voxelotor's ability to address the molecular basis of sickle cell disease (SCD) by reducing hemolytic anemia. These data support our goal of providing the sickle cell community with a potential new medicine that we believe could address the chronic damage and multi-organ dysfunction that all patients experience," said Ted W. Love, M.D., president and chief executive officer of GBT. "We are still on track for the potential acceptance of our New Drug Application for voxelotor in the second half of this year, in addition to the initiation of our post-approval transcranial Doppler (TCD) flow velocity confirmatory trial in the fourth quarter of 2019. In parallel, we are continuing our commercial preparations to ensure voxelotor is available to patients living with SCD across the country at the time of potential approval."

Recent Business Progress

Voxelotor

- Announced final agreement with the U.S. Food and Drug Administration on the design of the post-approval confirmatory study of voxelotor, utilizing TCD flow velocity as the primary endpoint.
- Simultaneously presented during the Presidential Symposium at the 2019 European Hematology Association (EHA) Annual Congress and published in *The New England Journal of Medicine* 24-week data from all participants enrolled in the Phase 3 HOPE Study. The study findings showed voxelotor provided a rapid, statistically significant and sustained improvement in hemoglobin levels and reduced the incidence of worsening anemia and hemolysis.
- Additional studies highlighted in poster presentations at the 2019 EHA Annual Congress included:
 - Clinical data from an investigator-initiated ancillary study of three adolescents with SCD enrolled in the HOPE-KIDS 1 Study showing unchanged or lower cerebral blood flow while receiving voxelotor, suggesting maintained or improved oxygen delivery to the brain; and
 - Results from an *in vitro* study of the mechanism of voxelotor demonstrating that voxelotor-modified hemoglobin releases oxygen under deoxygenated conditions and maintains oxygen delivery to tissues, supporting the safety of this investigational treatment.

Corporate

- Raised approximately \$197.8 million in net proceeds, after deducting underwriting costs and commissions and estimated offering expenses, from an underwritten public offering in June 2019 and related exercise of the over-allotment option in July 2019.
- Awarded more than \$200,000 in grants to five nonprofit organizations through the company's new Access to Excellent Care for Sickle Cell Patients Pilot Program (ACCEL). The program provides grant funding to support novel projects aimed at improving access to high-quality healthcare for individuals with SCD in the United States.
- Launched two SCD awareness campaigns, Sickle Cell Speaks, a national patient-focused campaign that aims to break down stigmas associated with the disease, and SCD Silent Damage, which seeks to help healthcare professionals increase their understanding of SCD and the resulting cascade of clinical complications leading to high levels of morbidity and mortality in patients.
- Expanded the management team with the appointment of Eric Fink as chief human resources officer. Fink is an accomplished leader with more than 10 years of experience building and leading multi-faceted human resources functions to support fast-growing biotechnology and pharmaceutical companies.

Financial Results for the Three Months Ended June 30, 2019

Cash, cash equivalents and marketable securities totaled \$731.7 million at June 30, 2019, compared with \$591.8 million at December 31, 2018.

Net loss for the three months ended June 30, 2019, was \$57.3 million compared with \$40.4 million for the same period in 2018. Basic and diluted net

loss per share for the three months ended June 30, 2019, was \$1.01 compared with \$0.78 for the same period in 2018. We continue to expect our net loss to significantly increase in the second half of 2019 as we expand our manufacturing efforts for voxelotor, commence the conduct of additional clinical studies of voxelotor in SCD, advance our pipeline programs, including inclacumab, 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 June 30, 2019, were \$36.0 million compared with \$31.6 million for the same period in 2018. The increase in R&D expenses is primarily attributable to increased employee-related costs and increased costs associated with our NDA submission activities. Total R&D non-cash stock compensation expense incurred for the three months ended June 30, 2019, was \$4.7 million compared with \$3.8 million for the same period in 2018.

G&A expenses for the three months ended June 30, 2019, were \$24.8 million compared with \$10.9 million for the same period in 2018. 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 buildout of the company's commercial operations. Total G&A non-cash stock compensation expense incurred in the three months ended June 30, 2019, was \$6.2 million, compared with \$4.0 million for the same period in 2018.

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 two therapies for the potential treatment of sickle cell disease, including its late-stage product candidate, voxelotor, as an oral, once-daily therapy. To learn more, please visit www.gbt.com and follow the company on Twitter [@GBT_news](https://twitter.com/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 the potential acceptance by the FDA of our rolling NDA for voxelotor under an accelerated regulatory approval pathway, 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-modifying therapy for SCD, our plan to initiate a post-approval TCD confirmatory study, our potential 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, 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 June 30, 2019, 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,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	\$ 36,010	\$ 31,573	\$ 70,476	\$ 61,517
General and administrative	24,794	10,914	42,849	23,665
Total operating expenses	60,804	42,487	113,325	85,182
Loss from operations	(60,804)	(42,487)	(113,325)	(85,182)
Other income (expense):				
Interest income, net	3,546	2,115	7,196	3,287
Other income (expenses), net	(63)	4	(115)	(29)
Total other income, net	3,483	2,119	7,081	3,258
Net loss	\$ (57,321)	\$ (40,368)	\$ (106,244)	\$ (81,924)
Basic and diluted net loss per common share	\$ (1.01)	\$ (0.78)	\$ (1.88)	\$ (1.65)
Weighted-average number of shares used in computing basic and diluted net loss per common share	56,539,760	51,742,904	56,386,560	49,767,633

GLOBAL BLOOD THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets

(In thousands)

	June 30, 2019 (Unaudited)	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 338,454	\$ 275,357
Short-term marketable securities	320,098	202,177
Prepaid expenses and other current assets	8,207	8,246
Total current assets	666,759	485,780
Property and equipment, net	12,345	14,981
Long-term marketable securities	73,176	114,281
Operating lease right-of-use assets	13,964	—
Other assets	2,588	2,601
Total assets	\$ 768,832	\$ 617,643
Liabilities and Stockholders' Equity		
Current liabilities	\$ 39,713	\$ 33,773
Operating lease liabilities, noncurrent	24,048	—
Other liabilities, noncurrent	881	11,071
Total liabilities	64,642	44,844
Total stockholders' equity	704,190	572,799
Total liabilities and stockholders' equity	\$ 768,832	\$ 617,643

Contact Information:

Stephanie Yao
 GBT
 650-741-7730
investor@gbt.com



Source: Global Blood Therapeutics, Inc.