



## GBT Reports Recent Business Progress and First Quarter 2020 Financial Results

May 6, 2020

*Achieved Oxbryta<sup>®</sup> (voxelotor) net revenues of \$14.1 million in first full quarter of launch*

*Approximately 1,650 new patient prescriptions for Oxbryta during the first quarter*

*Adapted to virtual operations and engagement intended to ensure ongoing access to Oxbryta during the COVID-19 pandemic*

*Contributed more than \$350,000 to support the immediate needs of sickle cell disease community during COVID-19*

*Conference call today at 4:30 p.m. ET*

**SOUTH SAN FRANCISCO, Calif., May 06, 2020 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the first quarter ended March 31, 2020.

"Our first quarter performance exceeded our expectations and demonstrates the quality of execution across our entire organization as well as our employees' commitment to deliver Oxbryta, a first-in-class therapy that directly inhibits the sickling and destruction of red blood cells. We have received positive feedback from patients and physicians, with increased awareness of Oxbryta and many patients responding well," said Ted W. Love, M.D., president and chief executive officer of GBT. "We are pleased that a growing number of sickle cell disease patients have started Oxbryta therapy and are enrolling in GBT Source, our patient support program. Our payer education activities are also going well, and we believe we are on track to meet our goal of obtaining broad payer coverage by the end of the year.

"With the emergence of the COVID-19 pandemic, we quickly adapted our business activities and launch tactics. We focused on ensuring our field teams were fully equipped to support our key stakeholders in an environment with restrictions on in-person meetings. We are encouraged that many healthcare professionals are embracing telemedicine to provide ongoing care for their patients. Additionally, in this challenging environment, I am proud that we expanded our support for the sickle cell disease community with direct donations to organizations providing immediate relief to patients and families across the country."

### Recent Business Progress

#### Commercial

- Achieved Oxbryta net sales of \$14.1 million in the first quarter of 2020.
- Recorded approximately 1,650 new patient prescriptions of Oxbryta during the first quarter, for a total of approximately 2,000 since launch. The vast majority of new patient prescriptions were enrollments into GBT Source™, the company's patient support program. The number of new patient prescriptions includes patients whose treatment was reimbursed and patients who received free medicine under GBT's patient assistance program due to insurance not providing coverage or not having insurance.
- Observed a decline in new patient prescriptions beginning in the second half of March due to the COVID-19 pandemic. GBT believes this decline is temporary and expects new patient prescriptions to return to pre-COVID levels once the pandemic subsides.
- Continued to put Oxbryta reimbursement coverage in place, with payers representing 25% of covered lives. GBT has secured coverage policies with Medicaid fee-for-service plans in 11 of 17 priority states. Most payer meetings to review Oxbryta remain on track for completion during the first half of 2020, and GBT believes it will meet the goal of obtaining broad coverage by the end of the year.

#### Clinical

- Paused new patient screening and enrollment in company-sponsored clinical trials due to the COVID-19 pandemic, while continuing to support patients already enrolled in studies and conduct study-related administrative activities. GBT anticipates restarting enrollment in these clinical trials at an appropriate time and currently does not expect this pause to impact long-term timelines for its clinical trials, including the completion of the HOPE-KIDS 2 post-approval confirmatory study.

#### Corporate

- Proactively took action intended to support public health efforts to address the COVID-19 pandemic and to ensure uninterrupted access and support for patients who are prescribed Oxbryta. This included temporarily suspending the company's field team from all in-person interactions to help slow the spread of the virus, and ensuring that GBT Source remained available and fully functional.
- Contributed more than \$350,000 to support the sickle cell disease (SCD) community during the COVID-19 pandemic through grants from the newly created GBT Community Fund to U.S. non-profit organizations, a donation to the Sickle Cell

Disease Association of America and personal contributions from GBT's board of directors and employees.

## Financial Results for the First Quarter 2020

Total product sales, net for the first quarter of 2020 was \$14.1 million, resulting from sales of Oxbryta. The company did not generate product sales in the first quarter of 2019.

Cost of sales for the three months ended March 31, 2020, was \$135,000. Manufacturing costs incurred prior to FDA approval of Oxbryta in November 2019 were previously recorded as research and development expense in the company's consolidated statement of operations. GBT expects that the cost of Oxbryta sales as a percentage of revenue will increase in future periods as product manufactured prior to FDA approval, and therefore fully expensed, is utilized. GBT did not incur cost of sales for Oxbryta in the first quarter of 2019 as no product sales were generated.

Research and development (R&D) expenses for the three months ended March 31, 2020, were \$39.8 million compared with \$34.5 million for the same period in 2019. The increase in R&D expenses for this comparative period was primarily attributable to increased costs related to preclinical research and manufacturing activities for inlacumab, increased employee-related costs, including non-cash stock compensation expense, and increased costs for other preclinical research activities related to the collaboration that GBT established with Syros Pharmaceuticals, Inc., in December 2019. The increase was partially offset by a decrease in manufacturing costs for Oxbryta. Following the approval of Oxbryta by the FDA in November 2019, GBT now capitalizes manufacturing of the product to inventory. Total R&D non-cash stock compensation expense incurred for the three months ended March 31, 2020, was \$5.4 million compared with \$4.0 million for the same period in 2019.

Sales, general and administrative (SG&A) expenses for the three months ended March 31, 2020, were \$47.7 million compared with \$18.1 million for the same period in 2019. The increase in SG&A expenses for this comparative period was primarily attributable to increased employee-related costs, including non-cash stock compensation expense, and increased professional and consulting services associated with the build-out of the company's commercial operations and launch of Oxbryta. Total SG&A non-cash stock compensation expense incurred in the three months ended March 31, 2020, was \$11.0 million compared with \$5.4 million for the same period in 2019.

Net loss for the three months ended March 31, 2020, was \$73.0 million compared with \$48.9 million for the same period in 2019. Basic and diluted net loss per share for the three months ended March 31, 2020, was \$1.20 compared with \$0.87 for the same period in 2019. The company expects its operating costs to increase in subsequent quarters due to costs associated with commercialization activities as well as costs associated with the advancement of its clinical pipeline.

Cash, cash equivalents and marketable securities totaled \$615.2 million at March 31, 2020, compared with \$695.0 million at December 31, 2019. The decrease of \$79.8 million from December 31, 2019 includes the one-time upfront payment of \$20 million paid in January 2020 related to the company's collaboration with Syros.

## Conference Call Details

GBT will host a conference call today, Wednesday, May 6, 2020, at 4:30 p.m. ET to provide a general business update and discuss the financial results for the first quarter 2020. To participate in the conference call, please dial 877-407-3982 (domestic) or 201-493-6780 (international). A live audio webcast of the conference call can be accessed on GBT's website at [www.gbt.com](http://www.gbt.com) under the Investors section. An archived audio webcast will be available for one month following the event.

## About Sickle Cell Disease

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa. It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.<sup>1</sup> SCD is a lifelong inherited blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.<sup>2</sup> Due to a genetic mutation, people with SCD form abnormal hemoglobin known as sickle hemoglobin. Through a process called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped and rigid.<sup>3-5</sup> The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.<sup>5-7</sup>

## About Oxbryta® (Voxelotor) Tablets

Oxbryta (voxelotor) is an oral, once-daily therapy for patients with sickle cell disease (SCD). Oxbryta works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes Oxbryta blocks polymerization and the resultant sickling and destruction of red blood cells. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that Oxbryta has the potential to modify the course of SCD. On November 25, 2019, Oxbryta received U.S. Food and Drug Administration (FDA) accelerated approval for the treatment of SCD in adults and children 12 years of age and older.<sup>7</sup> As a condition of accelerated approval, GBT will continue to study voxelotor in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of Oxbryta to decrease stroke risk in children 2 to 15 years of age.

In recognition of the critical need for new SCD treatments, the FDA granted Oxbryta Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD. The European Medicines Agency (EMA) has included voxelotor in its Priority Medicines (PRIME) program, and the European Commission (EC) has designated voxelotor as an orphan medicinal product for the treatment of patients with SCD.

## Important Safety Information

Oxbryta should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in Oxbryta. See the end of the patient leaflet for a list of the ingredients in Oxbryta.

Oxbryta can cause serious side effects, including serious allergic reactions. Patients should tell their healthcare provider or get emergency medical

help right away if they get rash, hives, shortness of breath or swelling of the face.

Patients receiving exchange transfusions should talk to their healthcare provider about possible difficulties with the interpretation of certain blood tests when taking Oxbryta.

The most common side effects of Oxbryta include headache, diarrhea, stomach (abdominal) pain, nausea, tiredness, rash and fever. These are not all the possible side effects of Oxbryta. Before taking Oxbryta, patients should tell their healthcare provider about all medical conditions, including if they have liver problems; if they are pregnant or plan to become pregnant as it is not known if Oxbryta can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbryta can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbryta and for at least 2 weeks after the last dose.

Patients should tell their healthcare provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements. Some medicines may affect how Oxbryta works. Oxbryta may also affect how other medicines work.

Patients are advised to call their doctor for medical advice about side effects. Side effects can be reported to FDA at 1-800-FDA-1088. Side effects can also be reported to Global Blood Therapeutics at 1-833-428-4968 (1-833-GBT-4YOU).

Full Prescribing Information for Oxbryta is available at [Oxbryta.com](http://Oxbryta.com).

## About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities. Founded in 2011, GBT is delivering on its goal to transform the treatment and care of sickle cell disease (SCD), a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta® (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, an underlying cause of SCD. GBT is also advancing its pipeline program in SCD with inclacumab, a p-selectin inhibitor in development to address pain crises associated with the disease. In addition, GBT's drug discovery teams are working on new targets to develop the next generation of treatments for SCD. To learn more, please visit [www.gbt.com](http://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 containing the words "will," "anticipates," "plans," "believes," "forecast," "estimates," "expects" and "intends," or similar expressions. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements 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. GBT intends these forward-looking statements, including statements regarding GBT's priorities, commitment, focus, goals and vision; the safety, efficacy and mechanism of action of Oxbryta and other product characteristics; the delivery, availability, use, and commercial and medical potential of Oxbryta; the commercialization of Oxbryta, including the activities and resources of the commercial field team and healthcare professionals; patient use of Oxbryta and GBT Source, including the recent decline in new patient prescriptions and related expectations; access and support for patients prescribed Oxbryta; payer education, meetings and coverage for Oxbryta; ongoing and planned studies of Oxbryta and related protocols, activities and expectations, including with respect to restarting enrollment and the impact on related timelines; GBT's financial position, outlook, guidance and expectations; transforming the treatment and care of SCD; and advancing GBT's pipeline, working on new targets and discovering, developing and delivering treatments, 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 GBT makes this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect GBT's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the company and on assumptions the company has made. GBT 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 GBT's control including, without limitation, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on GBT's business, including commercialization activities, regulatory efforts, research and development, corporate development activities, and operating results, which will depend on future developments that are highly uncertain and cannot be accurately predicted, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease; the risks that GBT has only recently established its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbryta; government and third-party payor actions, including those relating to reimbursement and pricing; risks and uncertainties relating to competitive products and other changes that may limit demand for Oxbryta; the risks regulatory authorities may require additional studies or data to support continued commercialization of Oxbryta; the risks that drug-related adverse events may be observed during commercialization or clinical development; data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval; compliance with the funding and other obligations under the Pharmakon loan; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, filed with the U.S. Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in GBT's subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, GBT assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.*

## References

1. Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). <https://www.cdc.gov/ncbddd/sicklecell/data.html>. Accessed June 3, 2019.
2. National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
3. Rees DC, et al. *Lancet*. 2010;376(9757):2018-2031.
4. Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.

5. Kato GJ, et al. *J Clin Invest.* 2017;127(3):750-760.
6. Caboot JB, et al. *Paediatr Respir Rev.* 2014;15(1):17-23.
7. Oxbryta (voxelotor) tablets prescribing information. South San Francisco, Calif. Global Blood Therapeutics, Inc.; November 2019.

**GLOBAL BLOOD THERAPEUTICS, INC.**

**Condensed Consolidated Statements of Operations**

(In thousands, except share and per share amounts)

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
	(Unaudited)	(Unaudited)
Product sales, net	\$ 14,118	\$ —
Costs and operating expenses:		
Cost of sales	135	—
Research and development	39,773	34,468
Selling, general and administrative	47,662	18,055
Total costs and operating expenses	<u>87,570</u>	<u>52,523</u>
Loss from operations	(73,452)	(52,523)
Other income (expense):		
Interest income	2,856	3,831
Interest expenses	(2,314)	(181)
Other expenses, net	(116)	(50)
Total other income, net	<u>426</u>	<u>3,600</u>
Net loss	<u>\$ (73,026)</u>	<u>\$ (48,923)</u>
Basic and diluted net loss per common share	<u>\$ (1.20)</u>	<u>\$ (0.87)</u>
Weighted-average number of shares used in computing basic and diluted net loss per common share	<u>60,787,710</u>	<u>56,231,587</u>

**GLOBAL BLOOD THERAPEUTICS, INC.**

**Condensed Consolidated Balance Sheets**

(In thousands)

	<b>March 31, 2020</b>	<b>December 31, 2019</b>
	(Unaudited)	
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 315,525	\$ 302,237
Short-term marketable securities	215,295	307,732
Other current assets	33,649	18,028
Total current assets	<u>564,469</u>	<u>627,997</u>
Property and equipment, net	37,329	27,113
Long-term marketable securities	84,378	85,030
Operating lease right-of-use assets	52,082	52,775
Other assets	2,888	3,184
Total assets	<u>\$ 741,146</u>	<u>\$ 796,099</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities	\$ 62,248	\$ 71,453
Long-term debt	73,688	73,559
Operating lease liabilities, noncurrent	81,638	72,359
Other noncurrent liabilities	—	34
Total liabilities	<u>217,574</u>	<u>217,405</u>
Total stockholders' equity	<u>523,572</u>	<u>578,694</u>

Total liabilities and stockholders' equity

\$ 741,146 \$ 796,099

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Source: Global Blood Therapeutics, Inc.