



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

November 5, 2020

*Achieved Oxbryta® (voxelotor) net revenues of \$36.9 million with more than 1,000 new patient prescriptions in the third quarter and net revenues of \$82.5 million through first nine months of 2020*

*New Oxbryta data and new research on GBT's pipeline to be presented at the 2020 American Society of Hematology Annual Meeting & Exposition in December*

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

**SOUTH SAN FRANCISCO, Calif., Nov. 05, 2020 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT), today reported recent business progress and financial results for the third quarter ended September 30, 2020.

"Our team continued to successfully execute on the launch of Oxbryta during the third quarter, expanding access and adoption of this first-in-class treatment that directly targets the underlying cause of sickle cell disease (SCD). We delivered an increase in new prescriptions despite a significant increase in COVID-19 infections in the U.S., including in states with the highest number of SCD patients – who are at high risk of serious illness from COVID-19. We also made substantial progress in adding new prescribers and expanding payer coverage, further establishing a strong foundation for the Oxbryta launch, about which we continue to be very pleased. We continue to monitor COVID-19 infection rates across the country and prioritize the health and safety of patients, healthcare providers, and our employees. We remain confident in the long-term potential of Oxbryta," said Ted W. Love, M.D., president and chief executive officer of GBT.

"During the quarter, we took another step toward making Oxbryta available globally with a distribution agreement aimed at providing access to the large number of sickle cell patients in the Middle East region," added Dr. Love. "As part of our ongoing commitment to raising awareness of the challenges facing SCD patients and improving their health outcomes, we co-hosted the 9<sup>th</sup> Annual Sickle Cell Disease Therapeutics Conference, furthering our efforts to support the SCD community. We look forward to reinforcing GBT's leadership in SCD at this year's ASH meeting, where we will present nine abstracts, including updates on the clinical profile of Oxbryta with longer-term data from the HOPE Study and several real-world experience abstracts. We are also excited to share new research on our pipeline as we continue to advance our goal of making sickle cell disease a well-managed condition."

### Recent Business Progress

#### Commercial

- Achieved Oxbryta (voxelotor) net sales of \$36.9 million and \$82.5 million in the three and nine months ended September 30, 2020, respectively.
- Recorded more than 1,000 new prescriptions of Oxbryta in the quarter, despite a dramatic increase in COVID-19 cases in the U.S. during the third quarter.
- The growth in new prescriptions from the second quarter reflects ongoing increases in the use of telemedicine by healthcare providers, virtual engagements with GBT field teams and in-person visits in some geographies. New prescriptions were higher in August and September, with some weeks nearing pre-pandemic levels. When the pandemic subsides, GBT expects that, over time, the number of new prescriptions will further improve and surpass pre-pandemic levels.
- Secured broad Oxbryta reimbursement coverage one quarter ahead of expectations, with 90% of lives covered by payers either through published policies or verified patient adjudication by the end of the third quarter. GBT has secured fee-for-service Medicaid coverage in 44 states, including all 17 priority states where most SCD patients live.

#### Clinical

- [Received](#) acceptance of nine abstracts on GBT's SCD programs to be presented at the 62<sup>nd</sup> American Society of Hematology (ASH) Annual Meeting & Exposition, which will be held on December 5-8. The presentations will include the 72-week analysis of the Phase 3 HOPE Study, real-world experience with Oxbryta, preclinical data highlighting the promise of the company's SCD pipeline, including inclacumab and the company's next generation hemoglobin S polymerization inhibitor.
- Announced that the company will host a virtual Analyst & Investor Day on December 7 to review data being presented at the 2020 ASH Annual Meeting.
- [Presented](#) two abstracts that provide greater insight into the safety and efficacy of Oxbryta at the 15th Annual Scientific Conference on Sickle Cell and Thalassemia (ASCAT) and 1st EHA European Sickle Cell Conference.

#### Corporate

- [Announced](#) an exclusive agreement with Biopharma-Middle East and Africa (Biopharma-MEA) to distribute Oxbryta in

Bahrain, Kuwait, Oman, Qatar, Saudi Arabia, and the United Arab Emirates, collectively known as the Gulf Cooperation Council (GCC) region. There are estimated to be more than 100,000 people age 12 years and older in this region living with SCD.<sup>1</sup>

- [Hosted](#) the 9th Annual SCD Therapeutics Conference, which highlighted recent advances and future trends in the treatment of SCD, and the impact of COVID-19 on this vulnerable patient population.
- Strengthened the company's leadership team with the appointment of Rajiv Patni, M.D., as chief medical officer.
  - Dr. Patni joined GBT from Portola Pharmaceuticals, where he was the chief medical officer. He brings 20 years of biopharmaceutical product development experience, including 16 programs and eight regulatory approvals, across the cardiology, diabetology, dermatology, hepatology, neurology, and hematology therapeutic areas.
- [Received](#) the 2020 Rare Impact Award<sup>®</sup> for Industry Innovation for Oxbryta from the National Organization for Rare Disorders (NORD); in addition, Oxbryta was selected as Breakthrough Drug of the Year by the 2020 National Xconomy Awards.

### **Financial Results for the Third Quarter 2020**

Total net product sales for the third quarter of 2020 was \$36.9 million, resulting from sales of Oxbryta. The company did not generate product sales in the third quarter of 2019.

Cost of sales for the three months ended September 30, 2020, was \$0.5 million. 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 third quarter of 2019 as no product sales were generated.

Research and development (R&D) expenses for the three months ended September 30, 2020, were \$40.2 million compared with \$39.1 million for the same period in 2019. The increase in R&D expense was primarily due to increased external costs related to GBT's inclacumab program and preclinical research activities related to the collaboration with Syros Pharmaceuticals, Inc., which were partially offset by decreased manufacturing costs for Oxbryta. Following FDA approval of Oxbryta in November 2019, GBT capitalizes manufacturing of Oxbryta to inventory. Total R&D non-cash stock compensation expense incurred for the three months ended September 30, 2020, was \$4.2 million compared with \$5.1 million for the same period in 2019.

Sales, general, and administrative (SG&A) expenses for the three months ended September 30, 2020, were \$54.5 million compared with \$29.7 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 September 30, 2020, was \$14.9 million compared with \$7.3 million for the same period in 2019.

Net loss for the three months ended September 30, 2020, was \$59.9 million compared with \$64.5 million for the same period in 2019. Basic and diluted net loss per share for the three months ended September 30, 2020, was \$0.97 compared with \$1.07 for the same period in 2019. The company expects its operating costs to increase in subsequent quarters due to costs associated with expanding commercialization activities as well as costs associated with the advancement of its clinical pipeline.

Cash, cash equivalents, and marketable securities totaled \$535.2 million on September 30, 2020, compared with \$695.0 million on December 31, 2019.

### **Conference Call Details**

GBT will host a conference call today, Thursday, November 5, 2020, at 4:30 p.m. ET to provide a general business update and discuss the financial results for the third quarter 2020. To participate in the conference call, please dial 877-252-3031 (domestic) or 312-281-1210 (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,<sup>2</sup> an estimated 52,000 people in Europe,<sup>3</sup> and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.<sup>2</sup> It also affects people of Hispanic, South Asian, Southern European, and Middle Eastern ancestry.<sup>2</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>4</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>4-6</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>5-8</sup>

### **About Oxbryta<sup>®</sup> (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, Oxbryta inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells. Through addressing hemolytic anemia and improving oxygen delivery throughout the body, 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>9</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 Oxbryta in its Priority

Medicines (PRIME) program, and the European Commission (EC) has designated Oxbryta as an orphan medicinal product for the treatment of patients with SCD.

GBT plans to seek regulatory approvals to expand the potential use of Oxbryta in the United States for the treatment of SCD in children age 4 to 11 years and to treat hemolytic anemia in SCD in people age 12 years and older in Europe.

### **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 two 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, the root cause of red blood cell sickling in 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, and GBT021601, the company's next generation hemoglobin S polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next wave of treatments for SCD. To learn more, please visit [www.gbt.com](http://www.gbt.com) and follow the company on Twitter @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, dedication, focus, goals, and vision; the safety, efficacy, and mechanism of action of Oxbryta, and other product characteristics; the commercialization, delivery, availability, and commercial and medical potential of Oxbryta; increases in use of telemedicine, virtual engagements and in-person visits; use of Oxbryta, including new prescriptions and prescribers and related expectations; payer coverage for Oxbryta; ongoing and planned studies of Oxbryta and related protocols, activities, and expectations; GBT's financial position, outlook, guidance, and expectations; the COVID-19 pandemic and related expectations; the potential expansion of the approved use of Oxbryta for more patients in the U.S. and potential approval of Oxbryta to treat patients in Europe; bringing Oxbryta to patients outside the U.S.; impacting the treatment, care and course of SCD; future presentations; the potential of GBT's pipeline, including inclacumab and other product candidates; 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 payer 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, and in GBT's most recent Quarterly Report on Form 10-Q 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

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3. European Medicines Agency. <https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182125>. Accessed June 12, 2020.
4. National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
5. Rees DC, et al. *Lancet*. 2010;376(9757):2018-2031.
6. Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.
7. Kato GJ, et al. *J Clin Invest*. 2017;127(3):750-760.
8. Caboot JB, et al. *Paediatr Respir Rev*. 2014;15(1):17-23.
9. 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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	(Unaudited)	(Unaudited)	(Unaudited)	(Unaudited)
Product sales, net	\$ 36,889	\$ —	\$ 82,508	\$ —
Costs and operating expenses:				
Cost of sales	513	—	1,025	—
Research and development	40,196	39,088	114,054	109,564
Selling, general and administrative	54,491	29,654	151,227	72,503
Total costs and operating expenses	95,200	68,742	266,306	182,067
Loss from operations	(58,311)	(68,742)	(183,798)	(182,067)
Other income (expense):				
Interest income	881	4,372	5,251	11,909
Interest expense	(2,291)	(146)	(6,887)	(487)
Other expenses, net	(160)	(31)	(313)	(146)
Total other income (expense), net	(1,570)	4,195	(1,949)	11,276
Net loss	\$ (59,881)	\$ (64,547)	\$ (185,747)	\$ (170,791)
Basic and diluted net loss per common share	\$ (0.97)	\$ (1.07)	\$ (3.04)	\$ (2.96)
Weighted-average number of shares used in computing basic and diluted net loss per common share	61,573,877	60,098,093	61,160,984	57,637,318

## GLOBAL BLOOD THERAPEUTICS, INC.

### Condensed Consolidated Balance Sheets

(In thousands)

	September 30, 2020	December 31, 2019
	(Unaudited)	
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 418,402	\$ 302,237
Short-term marketable securities	113,523	307,732
Other current assets	62,739	18,028

Total current assets	594,664	627,997
Property and equipment, net	38,697	27,113
Long-term marketable securities	3,275	85,030
Operating lease right-of-use assets	51,337	52,775
Other assets	3,291	3,184
Total assets	<u>\$ 691,264</u>	<u>\$ 796,099</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities	\$ 77,946	\$ 71,453
Long-term debt	73,880	73,559
Operating lease liabilities, noncurrent	80,439	72,359
Other noncurrent liabilities	1,344	34
Total liabilities	<u>233,609</u>	<u>217,405</u>
Total stockholders' equity	<u>457,655</u>	<u>578,694</u>
Total liabilities and stockholders' equity	<u>\$ 691,264</u>	<u>\$ 796,099</u>

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Source: Global Blood Therapeutics, Inc.