



GBT Reports Second Quarter 2021 Financial Results

August 3, 2021

Achieved Oxbryta[®] (voxelotor) net revenues of \$47.6 million, an increase of 51% year-over-year

Continued progress toward potential Oxbryta pediatric label expansion in U.S. and marketing authorization in Europe

Advanced pipeline with initiation of two inclacumab pivotal Phase 3 trials and GBT021601 (GBT601) Phase 1 trial

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

SOUTH SAN FRANCISCO, Calif., Aug. 03, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported financial results for the second quarter ended June 30, 2021 and recent business progress.

"In the second quarter we continued to grow the net number of patients on Oxbryta, reflecting the positive real-world experience reported by physicians and patients presented at major medical meetings and shown in our market research," said Ted W. Love, M.D., president and chief executive officer of GBT. "The fundamentals of the Oxbryta launch remain strong, and we have several initiatives ongoing and planned that are intended to drive awareness, adoption, and access. Although overall visits in the second quarter remained below pre-pandemic levels, we believe in-person reengagement will drive a gradual improvement in new prescriptions if the COVID-19 pandemic consistently improves."

"We believe GBT is well positioned for long-term success. As the body of real-world evidence for Oxbryta grows, we initiated multi-center, prospective and retrospective data registries that will track hematologic and clinical responses to treatment with the medicine. In addition, we submitted our applications seeking FDA approval of a pediatric formulation of Oxbryta in patients age 4 to 11, and we continue to execute our regulatory strategies in Europe and the Gulf Cooperation Council (GCC) countries in the Middle East," continued Dr. Love. "In addition, our R&D teams are advancing a portfolio of complementary product candidates that we believe could enhance care across a range of sickle cell disease (SCD) clinical manifestations. We initiated two pivotal Phase 3 studies for inclacumab and our Phase 1 study for GBT601 in SCD patients, giving us confidence that we will achieve our goal to present proof-of-concept results for GBT601 by the end of year."

Recent Business Progress

Commercial

- Achieved Oxbryta[®] (voxelotor) tablets net sales of \$47.6 million in the second quarter, an increase of 51% year-over-year. On a sequential basis, sales increased 22%, primarily driven by patient demand.
- Recorded approximately 925 new prescriptions for Oxbryta in the second quarter.
- The net number of patients taking Oxbryta increased compared to the prior quarter and has increased each quarter since launch.
- GBT believes that if the pandemic subsides in the second half of 2021, the number of new prescriptions will improve incrementally. In addition, GBT anticipates that in future periods new prescriptions will eventually surpass pre-COVID-19 levels.
- Most recent healthcare provider survey (n = 278, completed in June-July 2021) confirms continued high levels of satisfaction with Oxbryta, including its ability to keep SCD under control for patients. Nearly all prescribers aware of Oxbryta have or would prescribe it in the future.
- Oxbryta continues to have broad payer coverage, with more than 90% of covered lives having access through their healthcare plans.
- In July, launched the first branded direct-to-consumer television advertising campaign in sickle cell disease to educate and empower patients.

Clinical

- Presented new data from the Phase 2a HOPE-KIDS 1 Study at the European Hematology Association (EHA) 2021 Virtual Congress that showed children with SCD ages 4 to 11 years treated with Oxbryta achieved significant improvements in hemoglobin levels.
- Initiated the RETRO and PROSPECT data registries to enable deeper understanding of Oxbryta's long-term efficacy and safety. Initial results from the RETRO study were presented at EHA, demonstrating that after 12 months, 50% of patients had a greater than 1 g/dL increase in hemoglobin.
- Results from a single-center analysis including 77 patients (age 12-70) were presented at EHA, reinforcing the efficacy and safety of treatment with Oxbryta in a real-world setting, including with and without the use of hydroxyurea. In addition,

when measured by the patient and clinical global impressions of change scale, the majority of patients were rated much improved or very much improved.

- In July 2021, initiated two global, randomized, placebo-controlled, pivotal Phase 3 trials evaluating the safety and efficacy of inclacumab, GBT's P-selectin inhibitor, for the reduction of vaso-occlusive crisis (VOC) frequency and VOC-related hospital readmissions, respectively.
- Initiated a Phase 1 trial of GBT601 in SCD patients, with a goal of providing proof-of-concept data by the end of the year.
- Enrollment of pediatric [expanded access protocol](#) (EAP) is exceeding expectations and GBT plans to increase the number of patients from 50 to up to 150. The EAP is designed to provide access to Oxbritya prior to potential approval for children ages 4 to 11 years with SCD in the United States who have no alternative treatment options and are ineligible to participate in clinical trials of Oxbritya.

Corporate

- In July 2021, announced submission of a supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for Oxbritya for the treatment of SCD in children ages 4 to 11 years, together with a related separate NDA required to seek approval for a pediatric weight-based formulation of Oxbritya. GBT requested priority review for the sNDA and NDA, which, if granted, could result in a six-month review process. The FDA has a 60-day filing review period to determine whether the sNDA and the NDA are complete and acceptable for filing and review.
- Oxbritya became the first and only SCD treatment to receive the Promising Innovative Medicine (PIM) designation from the Medicines and Healthcare Products Regulatory Agency (MHRA) in the United Kingdom, where approximately 15,000 people live with SCD.¹
- In France, Oxbritya was granted Temporary Authorization for Use (cATU), providing early access to patients beginning in the third quarter 2021. There are an estimated 20,000 patients diagnosed with SCD in France.²
- In Germany, a compassionate use program for Oxbritya has been approved by the Federal Institute for Drugs and Medical Devices (BfArM). There are an estimated 3,000 patients diagnosed with SCD in Germany.³
- In the GCC countries, submitted and received validation of an application for approval of Oxbritya tablets in the United Arab Emirates (UAE).
- Awarded approximately \$450,000 in grants to U.S. community-based organizations and institutions to advance access to care for people living with SCD through the company's Access to Excellent Care for Sickle Cell Patients (ACCEL) program. The ACCEL program, which is in its third year and increased the total funding and number of grantees from 2020, provides support to accelerate the development of sustainable access-to-care programs for people living with SCD.
- Strengthened the company's leadership team with the appointment of industry veteran Carrie Krehlik as chief human resources officer, who brings more than 30 years of experience in human resources, primarily in the biopharmaceutical and technology industries. Previously, Ms. Krehlik served as senior vice president and chief human resources officer at Adicet Bio and in leadership roles at ZS Pharma, Hyperion Therapeutics, and InterMune.

Financial Results for the Second Quarter 2021

Total net product sales for the second quarter of 2021 were \$47.6 million, resulting from sales of Oxbritya, compared to \$31.5 million for the second quarter of 2020.

Cost of sales for the three months ended June 30, 2021, was \$0.7 million, compared with \$0.4 million for the same period in 2020. Manufacturing costs incurred prior to FDA approval of Oxbritya in November 2019 were previously recorded as research and development expense in the company's consolidated statement of operations. GBT expects the cost of Oxbritya sales as a percentage of revenue will increase in future periods as product manufactured prior to FDA approval, and therefore fully expensed, is completely utilized.

Research and development (R&D) expenses for the three months ended June 30, 2021, were \$51.8 million compared with \$34.1 million for the same period in 2020. The increase was primarily due to an increase in external costs related to the company's preclinical programs, and Oxbritya and inclacumab programs. Total R&D non-cash stock compensation expense incurred for the three months ended June 30, 2021, was \$4.9 million compared with \$3.4 million for the same period in 2020.

Sales, general, and administrative (SG&A) expenses for the three months ended June 30, 2021, were \$61.1 million compared with \$49.1 million for the same period in 2020. The increase in SG&A expense was primarily attributable to increased professional and consulting services associated with the company's commercial operations for Oxbritya and employee-related costs, including non-cash stock compensation expense. Total SG&A non-cash stock compensation expense incurred in the three months ended June 30, 2021, was \$15.1 million compared with \$13.1 million for the same period in 2020.

Net loss for the three months ended June 30, 2021, was \$69.6 million compared with \$52.8 million for the same period in 2020. Basic and diluted net loss per share for the three months ended June 30, 2021, was \$1.12 compared with \$0.86 for the same period in 2020. Second quarter loss per share included an anticipated increase in operating costs driven by expanding commercialization activities related to Oxbritya and the advancement of the company's clinical pipeline. GBT anticipates a sequential increase in operating expenses in the third quarter of 2021 as the company continues to ramp up these efforts.

Cash, cash equivalents, and marketable securities totaled \$437.4 million at June 30, 2021, compared with \$560.9 million at December 31, 2020.

Conference Call Details

GBT will host a conference call today, Tuesday, August 3, 2021, at 4:30 p.m. ET to discuss the financial results for the second quarter 2021 and provide a general business update. To participate in the conference call, please dial 877-407-3982 (domestic) or +1 201-493-6780 (international). A live audio webcast of the conference call can be accessed on GBT's website at 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,³ an estimated 52,000 people in Europe,⁴ and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.³ It also affects people of Hispanic, South Asian, Southern European, and Middle Eastern ancestry.³ SCD is a lifelong inherited rare blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.⁵ Due to a genetic mutation, individuals 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.⁵⁻⁷ 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.⁶⁻⁹

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, which are primary pathologies faced by every single person living with SCD. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that Oxbryta has the potential to modify the course of SCD. On Nov. 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.¹¹

As a condition of accelerated approval, GBT will continue to study Oxbryta in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of the therapy 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. Additionally, Oxbryta has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and the European Commission (EC) has designated Oxbryta as an orphan medicinal product for the treatment of patients with SCD. Also, in May 2021, Oxbryta was granted Promising Innovative Medicine (PIM) designation in the United Kingdom from the Medicines and Healthcare Products Regulatory Agency (MHRA).

The EMA has accepted for review GBT's Marketing Authorization Application seeking full marketing authorization of Oxbryta in the European Union to treat hemolytic anemia in SCD patients ages 12 years and older.

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.

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) tablets, 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 inlacumab, a P-selectin inhibitor in Phase 3 development to address pain crises associated with the disease, and GBT021601 (GBT601), 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 potential treatments for SCD. 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 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, mission, vision, and positioning; the safety, efficacy, and mechanism of action of Oxbryta, and other product characteristics; the commercialization, delivery, availability, use, and commercial and medical potential of Oxbryta, including the use, significance and potential of related initiatives; engagement with patients for Oxbryta, including impact of increased in-person engagement; significance of data presented at the EHA Congress; payer coverage for Oxbryta; the expanded access protocol for Oxbryta and other initiatives to provide early access, including the availability, enrollment, use and impact; ongoing and planned studies, clinical trials and registries, and related protocols, activities, timing, and other expectations; GBT's financial position, outlook, guidance, and expectations; the COVID-19 pandemic and related expectations, including the potential impact on prescriptions as the pandemic subsides; expanding access to Oxbryta, including related strategies, activities and expectations; regulatory submissions to potentially expand the approved use of Oxbryta for more patients and in a pediatric formulation in the U.S. and to treat patients in Europe and other territories, including potential review, timing and approval; the ACCEL Grant Program, including the related activities and expectations; impacting the treatment, care, and course of SCD and mitigating related complications; safety, efficacy, mechanism of action, potential and advancement of GBT's drug candidates and 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 is continuing to establish its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for research, development, manufacture, distribution, and commercialization activities; government and third-party payer actions, including those relating to reimbursement and pricing; risks and uncertainties relating to competitive treatments 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 obligations under the Pharmakon loan; and the timing and progress of activities under GBT's collaboration, license and distribution agreements; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2020, 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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GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations

(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Product sales, net	\$ 47,555	\$ 31,501	\$ 86,598	\$ 45,619
Costs and operating expenses:				
Cost of sales	748	377	1,332	512
Research and development	51,784	34,085	102,641	73,858
Selling, general and administrative	61,093	49,075	120,059	96,736

Total costs and operating expenses	113,625	83,537	224,032	171,106
Loss from operations	(66,070)	(52,036)	(137,434)	(125,487)
Other income (expense):				
Interest income	164	1,514	493	4,370
Interest expense	(3,677)	(2,282)	(7,366)	(4,596)
Other expenses, net	(9)	(36)	(215)	(153)
Total other income, net	(3,522)	(804)	(7,088)	(379)
Net loss	(69,592)	(52,840)	(144,522)	(125,866)
Basic and diluted net loss per common share	\$ (1.12)	\$ 0.86	\$ (2.32)	\$ (2.06)
Weighted-average number of shares used in computing basic and diluted net loss per common share	62,312,418	61,116,707	62,207,328	60,952,269

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(In thousands)

	June 30, 2021	December 31, 2020
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 419,311	\$ 494,766
Short-term marketable securities	18,085	66,126
Other current assets	87,463	71,271
Total current assets	524,859	632,163
Property and equipment, net	36,775	37,882
Operating lease right-of-use assets	49,421	50,722
Other assets	3,917	3,235
Total assets	\$ 614,972	\$ 724,002
Liabilities and Stockholders' Equity		
Current liabilities	\$ 72,675	\$ 79,032
Long-term debt	149,268	148,815
Operating lease liabilities, noncurrent	76,441	79,176
Other noncurrent liabilities	822	822
Total liabilities	299,206	307,845
Total stockholders' equity	315,766	416,157
Total liabilities and stockholders' equity	\$ 614,972	\$ 724,002

Contact:

Steven Immergut (media)
650-410-3258
simmergut@gbt.com

Courtney Roberts (investors)
650-351-7881
croberts@gbt.com



Source: Global Blood Therapeutics, Inc.