

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549**

FORM 8-K

CURRENT REPORT

**Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): May 5, 2021

GLOBAL BLOOD THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware
(State or Other Jurisdiction of Incorporation)

001-37539
(Commission File Number)

27-4825712
(I.R.S. Employer Identification No.)

181 Oyster Point Blvd.
South San Francisco, California 94080
(Address of Principal Executive Offices) (Zip Code)

(650) 741-7700
(Registrant's telephone number, including area code)

Not Applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	GBT	The NASDAQ Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02. Results of Operations and Financial Condition.

On May 5, 2021, Global Blood Therapeutics, Inc. reported recent business progress and financial results for the first quarter ended March 31, 2021. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in Item 2.02 of this Form 8-K (including Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No. **Description**

99.1	Press Release, dated May 5, 2021, furnished herewith
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Global Blood Therapeutics, Inc.

Date: May 5, 2021

By: /s/ Jeffrey Farrow
Jeffrey Farrow
Chief Financial Officer
(Principal Financial Officer)

GBT Reports First Quarter 2021 Financial Results

Achieved Oxbryta[®] (voxelotor) net revenues of \$39.0 million; on track with plans to potentially expand access to Oxbryta in the U.S. and Europe

Advanced pipeline programs inclacumab and GBT021601 (GBT601) and expanded pipeline with in-license of two novel small molecule programs

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

SOUTH SAN FRANCISCO, Calif., May 05, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today reported recent business progress and financial results for the first quarter ended March 31, 2021.

“In the first quarter, our team continued to drive adoption of Oxbryta among sickle cell patients and healthcare providers as the fundamentals of our launch remain strong. We introduced new and enhanced materials to educate physicians and patients about Oxbryta, as well as support adherence via new tools and starter kits to set up patients for success. Despite the headwinds we expected from the COVID-19 pandemic during the quarter, we continue to deliver new prescriptions, and the net number of patients on Oxbryta has increased each quarter since launch. As the year continues, we believe we will see a gradual improvement in new prescriptions as COVID-19 cases decrease, more people are vaccinated, and SCD patients begin to reengage with the healthcare system,” said Ted W. Love, M.D., president and chief executive officer of GBT.

“On the clinical front, we continue to see real-world evidence from patients taking Oxbryta that shows significant and sustained improvement in hemoglobin levels, reduction in hemolysis, and improved overall health status. The growing body of clinical evidence demonstrating the benefits of Oxbryta supports our belief that it should become a standard therapy in sickle cell disease. In addition, we are building an innovative pipeline to address SCD from multiple approaches. We are on track to start two Phase 3 trials of inclacumab by mid-year and to generate proof-of-concept data for GBT601 in SCD patients by year end, as we strive to make SCD a well-managed condition for patients,” added Dr. Love.

Recent Business Progress

Commercial

- Achieved Oxbryta[®] (voxelotor) tablets net sales of \$39.0 million in the three months ended March 31, 2021, an increase of 177% year-over-year. On a sequential basis, sales decreased 5% in the first quarter compared to the fourth quarter of 2020, driven primarily by lower inventory levels and a higher gross-to-net adjustment, partially offset by patient demand.
- Recorded approximately 950 new prescriptions of Oxbryta in the first quarter, despite new cases of COVID-19 reaching peak levels in the U.S. during this period.
- New prescriptions in the first quarter reflect fewer healthcare provider and patient interactions due to the increase in COVID-19 cases in the U.S. during this period, partially offset by utilization of telemedicine by healthcare providers. GBT continues to believe that when the pandemic subsides, the number of new prescriptions will improve and surpass pre-COVID-19 levels over time.
- Oxbryta continues to have broad payer coverage, with more than 90% of covered lives having access through their healthcare plans.
- Launched patient starter kits and new sales materials in the U.S. to provide deeper education on Oxbryta and increase patient adherence, and launched www.gbtsource.com, which provides information for patients, caregivers and health professionals on GBT Source Solutions[®] - the company’s dedicated sickle cell disease (SCD) patient support program that helps eligible patients start and stay on Oxbryta.
- A market research study of current Oxbryta users completed during the first quarter showed that 84% of participants reported that it works extremely well.

Clinical

- On track to initiate two global, randomized, placebo-controlled, pivotal Phase 3 trials evaluating the safety and efficacy of inclacumab by mid-year, and to begin studying GBT601 in SCD patients with a goal of providing proof-of-concept data by the end of the year.
- In April 2021, the complete 72-week results from the Phase 3 HOPE Study of Oxbryta were published in *The Lancet Haematology*. The results showed significant and sustained improvement in hemoglobin levels, reduction in hemolysis and improved overall health status in patients treated with Oxbryta.
- In April 2021, presented a poster at the Academy of Managed Care Pharmacy 2021 Meeting highlighting a large-scale, longitudinal analysis demonstrating that increased hemoglobin levels in adult patients with SCD significantly reduced the risk of developing new end-organ damage.
- In April 2021, results from a single-center analysis including 76 patients (age 12-70) with SCD were presented at the American Society of Pediatric Hematology/Oncology Conference, reinforcing the efficacy and safety of treatment with Oxbryta in a real-world setting. 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.

- Enrolled the first patient in the Phase 4 ActIVe study that is evaluating daily physical activity in SCD patients 12 years of age and older.

Corporate

- Received acceptance for review from the European Medicines Agency of Oxbryta's Marketing Authorization Application seeking full marketing approval to treat hemolytic anemia in patients with SCD 12 years of age and older.
- Remain on track to submit by mid-year to seek regulatory approval by the U.S. Food and Drug Administration to expand the Oxbryta label for treatment of SCD in children ages 4 to 11 years.
- Entered into an agreement with Sanofi S.A. to exclusively in-license worldwide rights to two early-stage research programs in SCD: one that pursues a novel anti-sickling mechanism and another that leverages a new approach to reduce inflammation and oxidative stress.
- On May 3, 2021, Kim Smith-Whitley, M.D. officially joined GBT as executive vice president and head of research and development.
- Strengthened the company's board of directors with the appointment of Alexis A. Thompson, M.D., M.P.H., a world-renowned hematologist and SCD expert, who brings decades of experience in clinical research, patient care, leadership and advocacy in hematology and will serve on the board's research and development committee.

Financial Results for the First Quarter 2021

Total net product sales for the first quarter of 2021 was \$39.0 million, resulting from sales of Oxbryta, compared to \$14.1 million for the first quarter of 2020.

Cost of sales for the three months ended March 31, 2021, was \$0.6 million, compared with \$0.1 million for the same period in 2020. 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 completely utilized.

Research and development (R&D) expenses for the three months ended March 31, 2021, were \$50.9 million compared with \$39.8 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, including an upfront payment related to the Sanofi in-license of two early-stage research programs in SCD, and the company's inclacumab program. Total R&D non-cash stock compensation expense incurred for the three months ended March 31, 2021, was \$4.9 million compared with \$5.4 million for the same period in 2020.

Sales, general and administrative (SG&A) expenses for the three months ended March 31, 2021, were \$59.0 million compared with \$47.7 million for the same period in 2020. The increase in SG&A expense was primarily attributable to increased employee-related costs, including non-cash stock compensation expense, and increased professional and consulting services associated with the company's commercial operations for Oxbryta. Total SG&A non-cash stock compensation expense incurred in the three months ended March 31, 2021, was \$15.1 million compared with \$11.0 million for the same period in 2020.

Net loss for the three months ended March 31, 2021, was \$74.9 million compared with \$73.0 million for the same period in 2020. Basic and diluted net loss per share for the three months ended March 31, 2021, was \$1.21 compared with \$1.20 for the same period in 2020. First quarter loss per share included an anticipated increase in operating costs driven by expanding commercialization activities related to Oxbryta and the advancement of the company's clinical pipeline. GBT anticipates a sequential increase in operating expenses in the second quarter of 2021 as the company continues to ramp up these efforts.

Cash, cash equivalents, and marketable securities totaled \$482.0 million at March 31, 2021, compared with \$560.9 million at December 31, 2020.

Conference Call Details

GBT will host a conference call today, Wednesday, May 5, 2021, at 4:30 p.m. ET to provide a general business update and discuss the financial results for the first quarter 2021. 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 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,¹ an estimated 52,000 people in Europe,² and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.¹ It also affects people of Hispanic, South Asian, Southern European, and Middle Eastern ancestry.¹ 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.³ 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.³⁻⁵ 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.⁴⁻⁷

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. 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 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.⁸

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.

The EMA has accepted for review GBT's Marketing Authorization Application seeking full marketing authorization of Oxbryta in Europe to treat hemolytic anemia in SCD patients ages 12 years and older. GBT also plans to seek regulatory approval to expand the potential use of Oxbryta in the United States for the treatment of SCD in children as young as 4 years old.

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), 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 (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 treatments for SCD. To learn more, please visit 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, use, adoption, and commercial and medical potential of Oxbryta; significance of patient starter kits, enhanced educational materials and related information in supporting

commercialization efforts; potential of Oxbryta as a standard therapy in SCD; payer coverage for Oxbryta; ongoing and planned studies and related protocols, activities, timing and other expectations; GBT's financial position, outlook, guidance, and expectations; the COVID-19 pandemic and related expectations; providing access to Oxbryta to more patients; regulatory submissions, review and approval to potentially expand the approved use of Oxbryta for more patients in the U.S. and to treat patients in Europe; impacting the treatment, care and course of SCD and making SCD a well-managed condition; the potential and advancement of GBT's pipeline, including inlacumab and other product candidates; 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 development, manufacture, distribution 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 obligations under the Pharmakon loan; and the timing and progress of activities under GBT's research collaborations; 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

- Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). <https://www.cdc.gov/ncbddd/sicklecell/data.html>. Accessed February 24, 2021.
- European Medicines Agency. <https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182125>. Accessed February 24, 2021.
- National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
- Rees DC, et al. *Lancet*. 2010;376(9757):2018-2031.
- Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.
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- 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 March	
	31,	
	2021	2020
	(Unaudited)	(Unaudited)
Product sales, net	\$ 39,043	\$ 14,118
Costs and operating expenses:		
Cost of sales	584	135
Research and development	50,857	39,773
Selling, general and administrative	58,966	47,662
Total costs and operating expenses	<u>110,407</u>	<u>87,570</u>
Loss from operations	(71,364)	(73,452)
Other income (expense):		
Interest income	329	2,856
Interest expenses	(3,689)	(2,314)

Other expenses, net	(206)	(116)
Total other income (expense), net	(3,566)	426
Net loss	<u>\$ (74,930)</u>	<u>\$ (73,026)</u>
Basic and diluted net loss per common share	<u>\$ (1.21)</u>	<u>\$ (1.20)</u>
Weighted-average number of shares used in computing basic and diluted net loss per common share	<u>62,101,070</u>	<u>60,787,710</u>

GLOBAL BLOOD THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(In thousands)

	<u>March 31, 2021</u>	<u>December 31, 2020</u>
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 445,310	\$ 494,766
Short-term marketable securities	36,736	66,126
Other current assets	76,086	71,271
Total current assets	<u>558,132</u>	<u>632,163</u>
Property and equipment, net	38,050	37,882
Operating lease right-of-use assets	50,085	50,722
Other assets	3,631	3,235
Total assets	<u>\$ 649,898</u>	<u>\$ 724,002</u>
Liabilities and Stockholders' Equity		
Current liabilities	\$ 58,967	\$ 79,032
Long-term debt	149,052	148,815
Operating lease liabilities, noncurrent	77,862	79,176
Other noncurrent liabilities	822	822
Total liabilities	<u>286,703</u>	<u>307,845</u>
Total stockholders' equity	<u>363,195</u>	<u>416,157</u>
Total liabilities and stockholders' equity	<u>\$ 649,898</u>	<u>\$ 724,002</u>

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