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): December 17, 2021

GLOBAL BLOOD THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-37539**	**27-4825712**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(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 8.01. Other Events.

On December 17, 2021, Global Blood Therapeutics, Inc. issued a press release titled "GBT's Oxbryta® (voxelotor) Receives Positive CHMP Opinion for the Treatment of Hemolytic Anemia in Patients with Sickle Cell Disease Aged 12 Years and Older." A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	**Description**
99.1	Press Release dated December 17, 2021
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: December 17, 2021

By: /s/ Jeffrey Farrow
Jeffrey Farrow
Chief Financial Officer

EXHIBIT 99.1

GBT's Oxbryta® (voxelotor) Receives Positive CHMP Opinion for the Treatment of Hemolytic Anemia in Patients with Sickle Cell Disease Aged 12 Years and Older

European Commission decision on marketing authorization anticipated first quarter of 2022

Oxbryta would be the first oral treatment available in Europe to directly inhibit sickle hemoglobin (HbS) polymerization, the underlying molecular cause of sickle cell disease

HbS polymerization and red blood cell sickling drive deterioration of red blood cell health and result in hemolysis and hemolytic anemia, leading to inflammation, organ damage and early death

SOUTH SAN FRANCISCO, Calif., Dec. 17, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending marketing authorization for Oxbryta® **(**voxelotor) tablets for the treatment of hemolytic anemia due to sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). Oxbryta, an oral treatment taken once daily, would be the first medicine available in Europe that directly inhibits sickle hemoglobin (HbS) polymerization, the molecular cause of sickling and destruction of red blood cells in SCD.

Based on this CHMP opinion, a decision by the European Commission (EC), which authorizes marketing approval in the European Union, is expected in the first quarter of 2022. If approved by the EC, Oxbryta will receive marketing authorization in all EU member states and Iceland, Liechtenstein and Norway.

"For far too long, people battling the devastating effects of sickle cell disease have had few therapeutic options. This positive CHMP opinion marks an important step forward to a new medicine for tens of thousands of people in Europe living with the disease," said Ted W. Love, M.D., president and chief executive officer of GBT. "By targeting the underlying cause, we believe Oxbryta has the potential to address severe complications experienced by patients throughout their life that ultimately lead to shortened life expectancy."

People living with SCD and those closest to them experience a lifelong journey with the disease that touches every aspect of their lives. Patients experience progressive, serious complications and morbidities, including end-organ damage, which lead to decreased quality of life and early mortality.[1] Furthermore, economic disadvantages and health inequalities experienced by many patients with SCD can have negative societal impacts in areas such as access to healthcare, education and employment.[2-8]

"The positive CHMP decision is of huge significance for the SCD community, and as a clinician I would welcome the availability of this new therapeutic approach for my patients," said Professor Mariane de Montalembert, co-head of the Necker Site for major sickle cell syndromes and other rare pathologies of red blood cells and erythropoiesis, Necker-Enfants Malades Hospital, Sickle Cell Center in Paris. "In clinical studies, voxelotor has demonstrated significant increases in hemoglobin levels and reductions in markers of hemolysis, which is expected to improve quality of life and, we hope, to reduce chronic organ damage associated with the disease."

The CHMP opinion is based on data demonstrating clinically meaningful and statistically significant improvements in hemoglobin (Hb) levels, accompanied by reductions in red blood cell destruction (hemolysis), for patients treated with Oxbryta. Data from the Phase 3 HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study of 274 patients 12 years of age and older with SCD showed that, after 24 weeks of treatment, 51.1% of patients receiving Oxbryta achieved a greater than 1 g/dL increase in Hb compared with 6.5% receiving placebo ($p<0.001$), with significant improvements in markers of hemolysis in indirect bilirubin and reticulocyte percentage.[9] Results from the HOPE Study were published in June 2019 in *The New England Journal of Medicine*. Oxbryta showed a favorable safety profile with limited and transitory adverse reactions. The most common adverse reactions occurring in ≥10% of patients treated with Oxbryta with a difference of >3% compared to placebo were headache (26% vs. 22%), diarrhea (20% vs. 10%), abdominal pain (19% vs. 13%), nausea (17% vs. 10%), fatigue (14% vs. 10%), rash (14% vs. 10%) and pyrexia (12% vs. 7%).[9]

The analysis of the complete data from the HOPE Study, published in *The Lancet Haematology* in April 2021, further demonstrated that Oxbryta, at a daily dose of 1,500 mg, resulted in rapid and durable improvements in Hb levels and markers of hemolysis over 72 weeks of treatment in SCD patients 12 years of age and older. At 72 weeks, 88.9% of patients receiving Oxbryta achieved a greater than 1 g/dL increase in Hb compared with 25% receiving placebo. In addition, at 72 weeks, 58.9% of patients receiving Oxbryta achieved a greater than 2 g/dL increase in Hb compared with 3.3% receiving placebo and 20.0% of patients receiving Oxbryta achieved a greater than 3 g/dL increase in Hb compared to 0% receiving placebo. The analysis also showed that study participants treated with Oxbryta had numerically fewer vaso-occlusive crises (VOCs), consistent with the trends at 24 weeks, and were three times less likely to experience an acute episode (decrease in Hb >2 g/dL from baseline). Treatment with Oxbryta was well tolerated, with no new safety or tolerability findings identified.[10]

Findings from a post-hoc analysis of the HOPE Study published in the *American Journal of Hematology* in January 2021 evaluated the incidence and outcomes of leg ulcers in SCD patients and further support the foundational role of HbS

polymerization inhibition in SCD treatment. Results of the analysis showed leg ulcers improved or resolved by week 72 in all patients (5 of 5) receiving Oxbryta 1,500 mg, compared with 63% of patients (5 of 8) in the placebo group.[11]

Oxbryta is currently approved in the United States for the treatment of SCD in adults and children 12 years of age and older.[12] GBT is seeking 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 of age. The Ministry of Health and Prevention (MOHAP) in the United Arab Emirates (UAE) has granted marketing authorization for Oxbryta for the treatment of SCD in adults and children 12 years of age and older.

About Sickle Cell Disease

Sickle cell disease (SCD) affects more than 100,000 people in the United States,[13] an estimated 52,000 people in Europe,[14] and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.[13] It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.[13] 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.[15] 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, crescent-shaped and rigid.[1, 15-16] The recurrent sickling process causes destruction of the red blood cells and 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 delivery throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.[1, 16-18] Complications of SCD begin in early childhood and can include neurocognitive impairment, acute chest syndrome, and overt stroke, among other serious issues.[19]

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, Oxbryta inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells leading to hemolysis and hemolytic anemia, 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. In November 2019, the U.S. Food and Drug Administration (FDA) granted accelerated approval for Oxbryta tablets for the treatment of SCD in adults and children 12 years of age and older.[12]

As a condition of accelerated approval in the United States, 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 14 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 was granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), Oxbryta was designated by the European Commission (EC) as an orphan medicinal product for the treatment of patients with SCD, and Oxbryta was granted Promising Innovative Medicine (PIM) designation in the United Kingdom from the Medicines and Healthcare products Regulatory Agency (MHRA).

FDA-Approved Indication

Oxbryta is a hemoglobin S polymerization inhibitor indicated for the treatment of sickle cell disease in adults and pediatric patients 12 years of age and older.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).[12]

FDA-Approved 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 (HbS) 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 HbS polymerization inhibitor. 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 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, dedication, commitment, focus, goals, mission and vision; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbryta; significance of the CHMP opinion for Oxbryta and potential and timing of a decision of the European Commission; ongoing and planned studies, clinical trials and registries and related protocols, activities, timing and other expectations; expanding access to Oxbryta, including related 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; altering the treatment, course and care of SCD and mitigating related complications; safety, efficacy, mechanism of action, advancement and potential of GBT's drug candidates and pipeline; and 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.

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Contact:

Steven Immergut (media)
+1 650 410 3258
simmergut@gbt.com

Claudia Nabaie (media Europe)
+41 79 906 5814
cnabaie@gbt.com

Courtney Roberts (investors)
+1 650 351 7881
croberts@gbt.com