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

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# FORM 8-K

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## CURRENT REPORT

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

**Date of Report (Date of earliest event reported): July 26, 2022**

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## GLOBAL BLOOD THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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| **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)

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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. ☐

In this report, "GBT," "Company," "we," "our," and "us" means Global Blood Therapeutics, Inc., and/or one or more of our subsidiaries, unless the context otherwise provides.

**Item 8.01. Other Events.**

On July 26, 2022, Global Blood Therapeutics, Inc. issued a press release titled "MHRA Grants Marketing Authorization for GBT's Oxbryta® (voxelotor) for Use in Great Britain for the Treatment of Hemolytic Anemia in Patients with Sickle Cell Disease Age 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 July 26, 2022 |
| 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: July 26, 2022

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

# MHRA Grants Marketing Authorization for GBT's Oxbryta® (voxelotor) for Use in Great Britain for the Treatment of Hemolytic Anemia in Patients with Sickle Cell Disease Age 12 Years and Older

## Voxelotor is the first medicine approved in Great Britain to directly inhibit sickle hemoglobin (HbS) polymerization, the underlying molecular cause of sickle cell disease

**SOUTH SAN FRANCISCO, Calif., July 26, 2022 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the Medicines and Healthcare products Regulatory Agency (MHRA) has granted Great Britain marketing authorization for Oxbryta® (voxelotor) for the treatment of hemolytic anemia due to sickle cell disease (SCD) in adult and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). Voxelotor, an oral treatment taken once daily, is the first medicine authorized in Great Britain that directly inhibits sickle hemoglobin (HbS) polymerization, the molecular basis of sickling and destruction of red blood cells in SCD.

"We welcome the MHRA's marketing authorization of voxelotor as a new treatment option for many people in Great Britain living with sickle cell disease, a devastating life-long condition which for far too long has seen little therapeutic innovation," said Beatriz F. Pujol, Ph.D., vice president, head of medical affairs EU & GCC at GBT. "Following this marketing authorization by the MHRA, we look forward to working with the National Institute of Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC) with the goal of helping to facilitate rapid access to voxelotor for people living with sickle cell disease who may benefit from this important treatment."

SCD affects approximately 15,000 people in the UK.[1] People living with SCD experience progressive, serious complications and morbidities, including organ damage, which lead to decreased quality of life and early mortality.[2] 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.[3-9]

In 2021, voxelotor was the first SCD treatment to receive a Promising Innovative Medicine (PIM) designation from the MHRA, which subsequently granted the medicine a positive scientific opinion under the Early Access to Medicines Scheme (EAMS). This enabled healthcare professionals to treat selected patients with voxelotor prior to market authorization based on clinical factors to address a clear unmet medical need.

The marketing authorization by the MHRA, which follows the European Commission (EC) authorization earlier this year, is based on results demonstrating clinically meaningful and statistically significant improvements in hemoglobin (Hb) levels, accompanied by a reduction of hemolysis markers, for patients treated with voxelotor. 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 voxelotor 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.[10] In the HOPE Study, 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%).[10] Results from the HOPE Study were published in June 2019 in *The New England Journal of Medicine* and the analysis of the complete data from the HOPE Study was published in *The Lancet Haematology* in April 2021.

The EC decision, which was granted in February 2022, provides marketing authorization in all EU member states, as well as the additional member states of the European Economic Area, including Iceland, Liechtenstein and Norway. The MHRA grants marketing authorization in Great Britain.

### About Sickle Cell Disease

It is estimated that more than 100,000 people in the United States,[11] 52,000 people in Europe,[12] up to 100,000 people in Brazil,[13] and millions of people throughout the world have sickle cell disease (SCD).[11] SCD occurs particularly among those whose ancestors are from sub-Saharan Africa, though it also occurs in people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.[11] 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.[14] Due to a genetic mutation, individuals with SCD form abnormal hemoglobin known as sickle hemoglobin. When sickle hemoglobin becomes deoxygenated, it polymerizes to form rods, which deforms the red blood cells into sickled – crescent-shaped, rigid – cells.[2,14,15] The recurrent sickling process causes destruction of the red blood cells, hemolysis and anemia (low hemoglobin due to red blood cell destruction), which drives vascular inflammation contributing to blockages in capillaries and small blood vessels (vaso-occlusion) that impede the flow of blood and oxygen delivery throughout the body. Episodes of painful vascular occlusions are commonly referred to as vaso-occlusive crises (VOCs). The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.[2,15-18] Complications of SCD begin in early childhood and can include neurocognitive impairment, acute chest syndrome, and silent and overt stroke, among other serious issues.[19] Early intervention and treatment of SCD have shown potential to modify the course of this disease, reduce symptoms and events, prevent long-term organ damage, and extend life expectancy.[2]

**About Oxbryta® (voxelotor)**
Voxelotor is an oral, once-daily therapy for patients with sickle cell disease (SCD). Voxelotor works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, voxelotor inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells leading to hemolysis and anemia, which are primary pathologies faced by every single person living with SCD. Through addressing hemolysis and anemia and improving oxygen delivery throughout the body, GBT believes that voxelotor has the potential to modify the course of SCD.

In November 2019, the U.S. Food and Drug Administration (FDA) granted accelerated approval for voxelotor tablets, under the brand name Oxbryta®, for the treatment of SCD in adults and children 12 years of age and older, and in December 2021, the FDA expanded the approved use of Oxbryta for the treatment of SCD in patients 4 years of age and older in the United States.[9] As a condition of accelerated approval for patients ages 4 and older in the United States, GBT is studying 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 received the prestigious 2021 Prix Galien USA award for "Best Biotechnology Product" from The Galien Foundation.

Oxbryta has been 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). In February 2022, the European Commission (EC) granted Marketing Authorization for Oxbryta for the treatment of hemolytic anemia due to SCD in adult and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). In addition, Oxbryta has been approved in the United Arab Emirates (UAE) and Oman for the treatment of SCD in adults and children 12 years of age and older.

Please click here for Important Safety Information and full Prescribing Information including Patient Information for Oxbryta in the U.S.

**About Global Blood Therapeutics**
Global Blood Therapeutics, Inc. (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities, starting with sickle cell disease (SCD). Founded in 2011, GBT is delivering on its goal to transform the treatment and care of SCD, a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta (voxelotor), the first FDA-approved medicine 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 Phase 3 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 wave of potential 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, vision and positioning; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, awareness, delivery, availability, use and commercial and medical potential of Oxbryta; significance of the marketing authorization for Oxbryta by the MHRA; working with NICE and SMC, including access to voxelotor and related timing; ongoing and planned studies, clinical trials and registries and related protocols, activities, timing and other expectations; altering the treatment, course, trajectory 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, 2021, 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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