



## **Oxbryta® (voxelotor) Tablets for Oral Suspension, A New Dispersible Tablet Dosage Form, Now Available for Patients with Sickle Cell Disease in the United States**

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**SOUTH SAN FRANCISCO, Calif., Jan. 10, 2022 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced Oxbryta® (voxelotor) tablets for oral suspension, a new dispersible, once-daily tablet dosage form recently approved by the U.S. Food and Drug Administration (FDA), is now available through GBT's specialty pharmacy partner network in the United States.

On December 17, 2021, [the FDA approved](#) Oxbryta (voxelotor) tablets for the treatment of sickle cell disease (SCD) in children ages 4 to less than 12 years – expanding the previously approved use of Oxbryta to treat SCD in patients ages 12 years and older in the U.S. The FDA also approved Oxbryta tablets for oral suspension, the new dispersible tablet dosage form suitable for patients ages 4 to less than 12 years. Oxbryta is the first and only approved medicine that directly targets sickle hemoglobin polymerization, the root cause of the sickling and destruction of red blood cells in SCD.

“The sickle cell disease community needs more innovative, safe and effective treatment options for this disease – a fact central to GBT’s mission,” said Kim Smith-Whitley, M.D., executive vice president and head of research and development at GBT. “The FDA’s recent approval of the new dispersible tablet dosage form of Oxbryta is an important advance for people and families affected by this devastating disease, and we are pleased to make this new dosage form available to patients by prescription. We are grateful to all those involved in ensuring that more sickle cell disease patients who need treatment with Oxbryta have access to it.”

Oxbryta is now available by prescription in two dosage forms for patients 4 years and older based on the patient’s age, weight, and ability to swallow tablets: 500 mg tablets and 300 mg tablets for oral suspension. The 300 mg dispersible tablets for oral suspension are available in bottles of 60 and 90 tablets. The dispersible tablets include grape flavoring and are intended to be dispersed in room-temperature clear drinks for ease of swallowing (such as drinking water or clear soda), making Oxbryta easier to take for patients who have difficulty swallowing whole tablets, particularly younger children.

GBT is committed to ensuring that patients who are prescribed Oxbryta can access their medicine. GBT Source Solutions® was established by GBT as a comprehensive program for patients who are prescribed Oxbryta that provides a wide range of practical, educational and financial support customized to each patient’s needs. GBT Source Solutions provides support by reviewing insurance coverage options and explaining benefits; explaining specialty pharmacy benefits and working with the specialty pharmacies that coordinate shipments of Oxbryta; helping eligible, commercially insured patients with co-pay assistance; and helping appropriate patients stay on treatment with a nurse support team. More information is available at [Oxbryta.com](#) or 1-833-428-4968 (1-833-GBT-4YOU).

### **About Sickle Cell Disease**

Sickle cell disease (SCD) affects more than 100,000 people in the United States,<sup>1</sup> an estimated 52,000 people in Europe,<sup>2</sup> and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.<sup>3</sup> It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.<sup>3</sup> 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.<sup>4</sup> 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.<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> Complications of SCD begin in early childhood and can include neurocognitive impairment, acute chest syndrome, and silent and overt stroke, among other serious issues.<sup>9</sup>

### **About Oxbryta® (voxelotor) Tablets and Tablets for Oral Suspension**

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, and in December 2021, the U.S. FDA expanded the approved use of Oxbryta for the treatment of SCD in patients 4 years of age and older.<sup>10</sup>

As a condition of accelerated approval for patients ages 4 and older 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 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 December 2021, the Committee for Medicinal Products for Human Use (CHMP) of the EMA adopted a positive opinion recommending marketing authorization for Oxbritya in Europe for the treatment of hemolytic anemia due to SCD in adults and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). The Ministry of Health and Prevention (MOHAP) in the United Arab Emirates (UAE) has granted marketing authorization for Oxbritya for the treatment of SCD in adults and children 12 years of age and older.

### **Important Safety Information**

Oxbritya should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in Oxbritya. See the end of the patient leaflet for a list of the ingredients in Oxbritya. Oxbritya 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 (difficult breathing) or swelling of the face.

The most common side effects of Oxbritya include headache, diarrhea, stomach-area (abdominal) pain, nausea, rash or hives, and fever. The most common side effects of Oxbritya in children ages 4 to less than 12 years of age include fever, vomiting, rash, stomach-area (abdominal) pain, diarrhea, and headache. These are not all the possible side effects of Oxbritya. Before taking Oxbritya, 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 Oxbritya can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbritya can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbritya 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 Oxbritya works. Oxbritya may also affect how other medicines work and may affect the results of certain blood tests.

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 Oxbritya is available at [Oxbritya.com](http://Oxbritya.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 Oxbritya<sup>®</sup> (voxelotor) tablets and tablets for oral suspension, 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](http://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, dedication, commitment, focus, goals, mission and vision; safety, efficacy and mechanism of action of Oxbritya and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbritya; ongoing and planned studies, clinical trials and registries, and related protocols, activities, timing and other expectations; significance of the FDA's approval of GBT's regulatory submissions to expand the approved use of Oxbritya for more patients and in a pediatric formulation in the U.S., including in advancing the treatment of SCD, the suitability, use and potential of such pediatric formulation and the impact on patients and others; GBT's commitment to ensuring access to Oxbritya, including the availability, use and impact of GBT Source Solutions; expanding the approved use of Oxbritya to treat patients in Europe and other territories, including potential regulatory 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 Oxbritya; 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 Oxbritya; the risks regulatory authorities may require additional studies or data to support continued commercialization of Oxbritya; 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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