



U.S. FDA Accepts for Priority Review Supplemental New Drug Application for Oxbryta® (voxelotor) for the Treatment of Sickle Cell Disease in Children Ages 4 to 11

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New Drug Application for Oxbryta dispersible tablets also accepted for priority review

Approximately 17,000 children ages 4 to 11 years have sickle cell disease in the United States

SOUTH SAN FRANCISCO, Sept. 07, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the U.S. Food and Drug Administration (FDA) has accepted for filing and review the company's supplemental New Drug Application (sNDA) seeking accelerated approval for Oxbryta® (voxelotor) for the treatment of sickle cell disease (SCD) in children ages 4 to 11 years and its New Drug Application (NDA) seeking approval for a new age-appropriate dispersible tablet dosage form of Oxbryta suitable for pediatric patients. Oxbryta, a first-in-class therapy that directly targets hemoglobin polymerization, the root cause of red blood cell sickling in SCD, is currently approved by the FDA in a tablet dosage form to treat SCD in patients age 12 years and older.

The FDA granted Priority Review for both the NDA and sNDA, providing a six-month review, and assigned a Prescription Drug User Fee Act (PDUFA) target action date for both applications of December 25, 2021.

"The FDA's acceptance of our regulatory submissions for Oxbryta for the treatment of sickle cell disease in children ages 4 to 11 years and a pediatric-friendly dosage form of Oxbryta is an important step toward achieving GBT's goal of bringing Oxbryta to all eligible patients suffering from this devastating disease," said Ted W. Love, M.D., president and CEO of GBT. "There are few current therapeutic options for children under 12 years of age with sickle cell disease, which can cause irreversible multi-organ damage in the first few years of life. Given the profound unmet need, we appreciate the FDA prioritizing the review of potential treatments for the long-overlooked sickle cell disease community."

In the U.S., approximately 17,000 children ages 4 to 11 years have sickle cell disease.¹ The Oxbryta pediatric sNDA and NDA are based on data from the open-label Phase 2a HOPE-KIDS 1 Study (GBT440-007). An [analysis of data](#) presented at the European Hematology Association (EHA) 2021 Virtual Congress in 45 children with SCD ages 4 to 11 years enrolled in the study showed that weight-based treatment with the Oxbryta dispersible tablets dosage form that is the subject of the NDA resulted in rapid and sustained improvements in hemoglobin. Concurrent reduction of hemolysis (or red blood cell destruction) was also demonstrated.²

The NDA seeks approval for 300 mg dispersible tablets. The dispersible tablet formulation includes grape flavoring, is intended to be dispersed in room-temperature drinking water or other clear drinks for ease of swallowing, and allows for weight-based dosing in pediatric patients ages 4 to 11 years with SCD.

Priority review is granted to therapies that the FDA determines have the potential to provide a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious condition. Under the PDUFA, a priority review targets a review time of six months compared to a standard review time of 10 months.

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,³ 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.⁴ 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.⁴⁻⁶ 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.⁵⁻⁸

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, 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 November 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.⁹

As a condition of accelerated approval, 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 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 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).

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

Important Safety Information about Oxbryta (voxelotor) tablets

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, 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 data presented at the EHA Congress, including support for the use of Oxbryta in children; ongoing and planned studies and clinical trials and related protocols, activities and expectations; expanding access to Oxbryta, including related activities and expectations; significance of the FDA's acceptance of GBT's regulatory submissions for Oxbryta; 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 suitability, use and potential of such pediatric formulation; altering the treatment, course and care of SCD and mitigating related complications; potential impact of addressing the root cause of red blood cell sickling at a young age; 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)
650-410-3258
simmergut@gbt.com

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



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