GBT’s Voxelotor is First Sickle Cell Disease Treatment to Receive Promising Innovative Medicine (PIM) Designation in the UK

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SOUTH SAN FRANCISCO, Calif. and LONDON, June 14, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT), a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments for underserved patient communities, announced today that the Medicines and Healthcare Products Regulatory Agency (MHRA) in the United Kingdom has granted a Promising Innovative Medicine (PIM) designation for voxelotor for the potential treatment of hemolytic anemia in adults and adolescent patients 12 years of age and older with sickle cell disease (SCD).

Following a review by the MHRA, PIM designations are given to promising treatments that are likely to offer a major advantage for patients and are an early indication that the treatment is a promising candidate for the Early Access to Medicines Scheme (EAMS). For the MHRA to grant a PIM designation, the product must meet each of the following three criteria:

- The condition should be life-threatening or seriously debilitating with high unmet need, meaning there is no method of treatment, diagnosis or prevention available, or existing methods have serious limitations.
- The medicinal product is likely to offer major advantage over methods currently used in the UK, based on both non-clinical and clinical data.
- The potential adverse effects of the medicinal product are likely to be outweighed by the benefits, allowing for the reasonable expectation of a positive benefit risk balance.¹

SCD is a rare genetic condition which affects approximately 15,000 people in the UK² and 52,000 people across Europe.³ It has a devastating impact on the lives of those it affects and their families, including serious and life-threatening complications that can lead to organ damage and early death. Despite SCD being the first genetic disease to be examined at a molecular level,⁴ there remains a lack of urgency to treat SCD, which traditionally affects the African and Caribbean community, and healthcare disparities based on race persist.

“The sickle cell disease community, which for decades has been dramatically underserved, deserves innovative treatments that address the underlying cause of this debilitating disease,” said Nigel Nicholls, UK general manager of GBT. “Voxelotor is the first SCD treatment to receive the PIM designation, and this is a significant milestone in our efforts to potentially make this therapy available in the UK. This is an important step forward on our journey, and we remain committed to developing novel treatments with the hope of transforming the lives of those living with SCD.”

A first-in-class oral, once-daily therapy, voxelotor directly inhibits hemoglobin S polymerization, the root cause of the sickling and destruction of red blood cells in SCD. The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction), which impairs adequate oxygen delivery to the tissues and organs in the body.

Voxelotor is approved in the United States under the trade name Oxbryta® for the treatment of SCD in patients ages 12 years and older. GBT previously announced its plans to seek regulatory approval from the European Medicines Agency (EMA) for voxelotor in the treatment of hemolytic anemia in SCD patients ages 12 years and older.

Prior to potential marketing authorization, GBT initiated an early access program for voxelotor in Europe and other regions outside the United States, which enables physicians to use early access regulatory and legal pathways to request voxelotor for the treatment of hemolytic anemia in eligible patients with SCD who do not have access to the medicine as part of a clinical trial. If approved for EAMS, the voxelotor early access program would be further extended to eligible UK patients.

About Sickle Cell Disease

SCD is a rare genetic condition which affects approximately 15,000 people in the UK², 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.⁵ It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.⁵ 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.⁶ 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.⁶ ⁸ 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.⁷ ¹⁰

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.11

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. Oxbryta was granted Promising Innovative Medicine (PIM) designation in Great Britain 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. 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 health care 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 health care 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 health care 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 two weeks after the last dose.

Patients should tell their health care 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 the 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).

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) in the United States, 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 GTB021601 (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, dedication, commitment, focus, goals, mission and vision; safety, efficacy and mechanism of action of Oxbryta (or voxelotor) 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 voxelotor’s designation as a PIM; ongoing and planned studies and related protocols, activities and expectations; 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; the early access program for voxelotor, including the framework, availability, use and impact; altering the treatment, course and care of SCD, mitigating related complications and transforming the lives of people with SCD; potential and advancement of GBT’s pipeline, including inclacumab and other product candidates; 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 development, manufacture, distribution and commercialization...
activities related to Oxbryta; government and third-party payor 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 collaborative, 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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