



GBT Initiates Early Access Program for Voxelotor in Patients with Sickle Cell Disease in Europe and Other Regions Outside the United States

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SOUTH SAN FRANCISCO, Calif., Dec. 02, 2020 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced the initiation of an early access program for voxelotor, in Europe and other regions outside the United States, for the treatment of hemolytic anemia in sickle cell disease (SCD) patients ages 12 years and older.

"With no currently approved therapies outside the United States to treat hemolytic anemia in sickle cell disease, the unmet need in this devastating disease is profound," said Ted W. Love, M.D., president and chief executive officer of GBT. "As part of our deep, long-term commitment to patient access worldwide, we are pleased to initiate this early access program for eligible patients who may benefit from voxelotor."

A first-in-class oral, once-daily therapy, voxelotor directly inhibits hemoglobin 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](#) it plans to seek regulatory approval to treat hemolytic anemia in SCD in patients ages 12 years and older in Europe.

An early access program is a mechanism to make medicines available pre-approval upon request by a physician for appropriate patients with no alternative treatment option.

Through the voxelotor early access program, physicians in countries with an early access regulatory and legal pathway may be able to request voxelotor for eligible SCD patients who do not have access to the medicine as part of a clinical trial.

The program is being implemented in partnership with and administered by Inceptua Group's Medicines Access division, which has expertise in the strategy, design, and operational implementation of pre-approval access programs that make pharmaceutical products in clinical development available to patients as appropriate.

All requests must be submitted by the treating physician on behalf of the eligible SCD patient. Healthcare providers can obtain details about the voxelotor early access program by contacting Inceptua at access@inceptua.com.

For the six Middle Eastern countries in the Gulf Cooperation Council (GCC), GBT previously [announced](#) an exclusive distributorship that references the U.S. approval of Oxbryta to allow for access to the medicine while health authorities conduct their regulatory reviews, thereby obviating the need for this new early access program.

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,² 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.³ 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.³⁻⁵ 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.⁴⁻⁷

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. With the potential to improve hemolytic anemia and oxygen delivery, 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.⁸ 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.

GBT plans to seek regulatory approvals to expand the potential use of Oxbryta in the United States for the treatment of SCD in children ages 4 to 11 years and to treat hemolytic anemia in SCD in patients ages 12 years and older in Europe.

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), 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 GBT021601, 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](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, and vision; the safety, efficacy, and mechanism of action of Oxbryta and other product characteristics; the commercialization, delivery, availability, use, and commercial and medical potential of Oxbryta; ongoing and planned studies of Oxbryta and related protocols, activities, and expectations; the potential expansion of the approved use of Oxbryta for more patients in the U.S., and potential regulatory approval for Oxbryta to treat patients in Europe; the need for approved therapies outside the United States to treat hemolytic anemia; an early access program for voxelotor, including the potential initiation, framework, availability, use and impact; altering the treatment, course, and care of SCD and alleviating related complications; the commitment to patient access to treatment for SCD; the distributorship for the Gulf Cooperation Council countries, including access to voxelotor during regulatory review; the potential of GBT's pipeline, including inclacumab and other product candidates; and advancing GBT's pipeline, 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 the obligations under the Pharmakon loan; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, 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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