



FDA Approves Oxbryta™ (Voxelotor), the First Medicine Specifically Targeting the Root Cause of Sickle Cell Disease

November 25, 2019

First and only FDA-approved sickle hemoglobin polymerization inhibitor, a new class of therapy

Oxbryta broadly indicated for the treatment of sickle cell disease in adults and children 12 years of age and older¹

*Approval comes three months ahead of FDA's priority review action date,
allowing for quicker patient access to Oxbryta*

GBT to host conference call today, November 25, at 4:30 p.m. ET/1:30 p.m. PT

SOUTH SAN FRANCISCO, Calif., Nov. 25, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the U.S. Food and Drug Administration (FDA) has granted accelerated approval for Oxbryta™ (voxelotor) tablets for the treatment of sickle cell disease (SCD) in adults and children 12 years of age and older.¹ Oxbryta, an oral therapy taken once daily, is the first approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of SCD.^{2,3} The medicine is expected to be available through GBT's specialty pharmacy partner network within two weeks.

"Today is a major milestone not only for GBT but, most importantly, for people living with SCD, their families and those who care for them. When we started our journey with the SCD community more than eight years ago, we set out to transform the way this devastating, lifelong disease is treated," said Ted W. Love, M.D., president and chief executive officer of GBT. "We are proud to bring this breakthrough therapy to the SCD community. Uniquely developed from inception to treat SCD, Oxbryta embodies GBT's commitment to develop and deliver innovative medicines for patients with overlooked, life-limiting chronic diseases. We are grateful to the patients, caregivers, clinical trial investigators, healthcare providers and advocates who have worked alongside us to develop this first-in-class therapy."

SCD affects an estimated 100,000 people in the United States and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa. It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.⁴ 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.² Due to a genetic mutation, people 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.^{2,3,5} 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.⁵⁻⁷

"Every person with SCD experiences hemoglobin polymerization and suffers from varying severity of anemia and hemolysis," said Elliott Vichinsky, M.D., director of hematology/oncology at UCSF Benioff Children's Hospital in Oakland, California. "With today's approval of Oxbryta, we now have a therapy that significantly improves hemoglobin levels, has a favorable safety profile and reduces the anemia and hemolysis that inevitably leads to the long-term and often undetected detrimental effects associated with this chronic genetic condition."

The accelerated approval of Oxbryta is based on clinically meaningful and statistically significant improvements in hemoglobin levels, accompanied by reductions in red blood cell destruction (hemolysis). 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 Oxbryta achieved a greater than 1 g/dL increase in hemoglobin compared with 6.5% receiving placebo (p<0.001). Results from the HOPE Study were published in June 2019 in [The New England Journal of Medicine](#).⁸

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%).¹

"SCD is a devastating, lifelong, inherited blood disorder that greatly impacts a person's life, including their ability to work, attend school and look after their families, and it can reduce their overall life expectancy," said Beverley Francis-Gibson, president and CEO of the Sickle Cell Disease Association of America. "After decades of waiting, we now have a treatment option that could change the course of this disease. We look forward to continuing to collaborate with GBT on initiatives aimed at transforming the care of patients living with SCD and ensuring access to important and innovative new medicines."

GBT is committed to ensuring that people with SCD who are prescribed Oxbryta have help accessing the medicine. The company has established GBT Source™, 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 provides support by reviewing insurance coverage options and explaining benefits; working with the specialty pharmacy partner network to coordinate delivery of Oxbryta to wherever the patient chooses; helping with financial and co-pay assistance for eligible patients; and helping patients stay on treatment as prescribed by their treating physicians with a nurse support team. More information is available at www.Oxbryta.com or 1-833-428-4968 (1-833-GBT-4YOU).

The FDA instituted its accelerated approval pathway to allow for earlier approval of drugs that treat serious conditions and that fill an unmet medical need based on a surrogate endpoint.⁹ As a condition of accelerated approval, GBT will continue to study Oxbritya in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial doppler (TCD) flow velocity to demonstrate a decrease in stroke risk in children 2 to 15 years of age. The study will be initiated by the end of the year.

In recognition of the critical need for new SCD treatments, the FDA reviewed Oxbritya under Priority Review and granted Oxbritya Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD.

Conference Call Details

GBT's management team will host a conference call and audio webcast today, November 25, at 4:30 p.m. ET/1:30 p.m. PT to discuss the FDA's accelerated approval of Oxbritya. To access the conference call, dial 844-471-0808 (U.S.) or 480-696-7309 (international) and refer to conference ID 5879959. The live audio webcast can be accessed in the Investor Relations section of the GBT website at www.gbt.com, and a replay will be available for 30 days following the call.

Indication

Oxbritya is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older. It is not known if Oxbritya is safe and effective in children below 12 years of age.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

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 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 Oxbritya.

The most common side effects of Oxbritya include headache, diarrhea, stomach (abdominal) pain, nausea, tiredness, rash and fever. 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.

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 https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/213137s000lbl.pdf.

About GBT

GBT is a biopharmaceutical company determined to discover, develop and deliver innovative treatments that provide hope to underserved patient communities. The company has one FDA-approved therapy for sickle cell disease and one investigational therapy in development for the disease. 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 the significance of the FDA approval of Oxbritya, the safety, efficacy and mechanism of action of Oxbritya and other product characteristics, the availability, use, commercialization and commercial and medical potential of Oxbritya, the therapeutic potential and safety profile of voxelotor for SCD, including the potential to be a disease-modifying therapy, to transform the treatment paradigm and to become a new standard of care, implementing and completing clinical development plans for voxelotor, generating and reporting data and analyses from past, ongoing and potential future studies of voxelotor, GBT's commercial infrastructure and manufacturing efforts, and developing and delivering innovative medicines, 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, the risks that GBT has only recently begun establishing its salesforce and other commercialization capabilities and may not be able to successfully launch or commercialize Oxbritya, risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbritya, government and third-party payor actions, including relating to reimbursement and pricing, risks and uncertainties relating to competitive products 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, and data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, along with those risks set forth in GBT's Annual Report

on Form 10-K for the fiscal year ended December 31, 2018, 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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