



GBT Receives Rare Impact Award for Innovation from the National Organization for Rare Disorders (NORD) for Development of Oxbryta® (voxelotor) Tablets.

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SOUTH SAN FRANCISCO, Calif., Oct. 12, 2020 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) announced today it has received the [2020 Rare Impact Award® for Industry Innovation](#) from the National Organization for Rare Disorders (NORD). GBT received the award for Oxbryta® (voxelotor) tablets at a virtual event on October 9, 2020, following NORD's annual Rare Disease and Orphan Products Breakthrough Summit.

Discovered and developed by GBT, Oxbryta is the first and only therapy that directly inhibits hemoglobin polymerization, the root cause of the sickling and destruction of red blood cells in sickle cell disease (SCD). It is currently approved in the United States to treat SCD in adults and adolescents age 12 years and older.

"We are honored to receive this recognition from NORD for developing novel therapeutics that meet the needs of the sickle cell community, which has been underserved for far too long," said Ted W. Love, M.D., president and CEO of GBT. "The true winners are sickle cell disease patients and their families, who now have more available treatment options. We look forward to continuing to work closely with healthcare providers, patients, and the rare disease community, whose partnership was integral to the development of Oxbryta, to advance our shared goal of making a difference in the lives of people with this devastating, inherited disease."

The NORD Rare Impact Awards celebrate the individuals, groups, and companies making extraordinary contributions to the lives of rare disease patients and caregivers. NORD is the leading independent advocacy organization committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

In addition, on September 24, 2020, Oxbryta was selected by the [2020 National Xconomy Awards as Breakthrough Drug of the Year](#). Each year, the National Xconomy Awards honor the top individuals, companies, and organizations within the United States life sciences ecosystem.

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 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, 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.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, Oxbryta inhibits sickle hemoglobin polymerization and the resultant sickling and destruction of red blood cells. 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.⁸ As a condition of accelerated approval, GBT will continue to study voxelotor in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of Oxbryta 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. The European Medicines Agency (EMA) has included Oxbryta in its Priority Medicines (PRIME) program, 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 age 4 to 11 years and to treat hemolytic anemia in SCD in people age 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 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).

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. In addition, GBT's drug discovery teams are working on new targets to develop the next generation 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).

References

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