



GBT Announces Upcoming Data Presentations at European Hematology Association (EHA) 2021 Virtual Congress

May 12, 2021

New Oxbryta® (voxelotor) Data from Phase 2a HOPE-KIDS 1 Study Demonstrate Improvement in Hemoglobin and Hemolysis in Children Ages 4 to 11 Years with Sickle Cell Disease

SOUTH SAN FRANCISCO, Calif., May 12, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that six abstracts related to its sickle cell disease (SCD) programs will be presented at the [European Hematology Association \(EHA\) 2021 Virtual Congress](#), taking place online June 9-17, 2021. Results from an analysis of the Phase 2a HOPE-KIDS 1 Study (GBT440-007) in children with SCD ages 4 to 11 years treated with Oxbryta® (voxelotor) tablets will be presented for the first time in an oral session.

The HOPE-KIDS 1 analysis, which evaluated 45 children treated with Oxbryta (1,500 mg or weight-based equivalent), found that 47 percent achieved a hemoglobin increase of >1 g/dL at 24 weeks. Reductions in markers of hemolysis were also observed. Oxbryta was well tolerated, and no new adverse safety signals were detected. The most commonly reported treatment-related adverse events were diarrhea (11 percent), vomiting (11 percent) and rash (11 percent).

These findings from the HOPE-KIDS 1 Study are consistent with data from the Phase 3 HOPE Study of patients with SCD ages 12 years and older, which was published in [The New England Journal of Medicine](#) and presented as part of the Presidential Symposium at the EHA Congress in June 2019. The complete analysis of 72-week data from the HOPE Study was recently published in [The Lancet Haematology](#), showing durable improvement in hemoglobin levels and reduction in hemolysis for patients receiving Oxbryta.

"New treatment options for younger children with sickle cell disease are urgently needed. We believe that reducing the sickling and destruction of red blood cells, thereby improving the anemia and hemolysis that characterize this devastating inherited condition early in life, could modify the course of the disease and alleviate serious and life-threatening complications," said Ted W. Love, M.D., president and chief executive officer of GBT. "We are encouraged by the extensive body of new data being presented at this year's EHA Congress, supporting the use of Oxbryta in both real-world and clinical trial settings. We look forward to potentially expanding access to Oxbryta over time across a broad range of sickle cell disease patients in the U.S., Europe, the Gulf Cooperation Council region and other areas."

The European Medicines Agency (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.

The EHA abstracts are available at <https://ehaweb.org>. All oral and poster presentations will be available on the EHA website on Friday, June 11, at 9:00 a.m. CEST/3:00 a.m. EDT. Other abstracts to be presented include real-world evidence supporting the use of Oxbryta in patients ages 12 and older. Full details are as follows:

Oral Session: Changing the scene on sickle cell disease

Abstract #S260: Safety and Efficacy of Voxelotor in Pediatric Patients with Sickle Cell Disease Aged 4-11 Years

Presenter: Clark Brown, M.D., Ph.D., Children's Healthcare of Atlanta

Live Q&A: Tuesday, June 15, 16:00-16:45 CEST/10:00-10:45 a.m. EDT

Poster Session: Sickle cell disease

Abstract #EP1209: Real-World Experience of Voxelotor for the Treatment of Patients with Sickle Cell Disease – A Single-Center Study

Presenter: Alan R. Anderson, M.D., University of South Carolina School of Medicine Greenville

Abstract #EP1206: Real-World Experience of Patients with Sickle Cell Disease Treated with Voxelotor: A Multicenter, Retrospective Study

Presenter: Biree Andemariam, M.D., University of Connecticut Health

Abstract #EP1198: GBT021601 Improves the Pathophysiology of Sickle Cell Disease in a Murine Model

Presenter: Kobina Dufu, Ph.D., GBT

Abstract #EP1201: Silent Cerebral Infarcts in Pediatric Sickle Cell Disease Natural History Cohort: Too Many, Too Soon?

Presenter: Raffaella Colombatti, M.D., Ph.D., University of Padova, Italy

Publication Only

Abstract #PB1769: Pediatric Patient Reported Outcomes in Patients Receiving Voxelotor for Sickle Cell Disease

First Author: Clark Brown, M.D., Ph.D., Director of Sickle Cell Clinical Research, Children's Healthcare of Atlanta

About Sickle Cell Disease

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States,² 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.² It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.² 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 Oxbrya® (voxelotor) tablets

Oxbrya (voxelotor) is an oral, once-daily therapy for patients with sickle cell disease (SCD). Oxbrya works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, Oxbrya 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 Oxbrya has the potential to modify the course of SCD. On Nov. 25, 2019, Oxbrya 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 Oxbrya 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 Oxbrya Breakthrough Therapy, Fast Track, Orphan Drug, and Rare Pediatric Disease designations for the treatment of patients with SCD. Additionally, Oxbrya has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and the European Commission (EC) has designated Oxbrya as an orphan medicinal product for the treatment of patients with SCD.

Important Safety Information

Oxbrya should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in Oxbrya. See the end of the patient leaflet for a list of the ingredients in Oxbrya. Oxbrya 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 Oxbrya.

The most common side effects of Oxbrya include headache, diarrhea, stomach (abdominal) pain, nausea, tiredness, rash and fever. These are not all the possible side effects of Oxbrya.

Before taking Oxbrya, 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 Oxbrya can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbrya can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbrya 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 Oxbrya works. Oxbrya 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 Oxbrya is available at Oxbrya.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 Oxbrya® (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 inclacumab, a P-selectin inhibitor in 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 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 Oxbrya and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbrya; significance of data to be presented at the EHA Congress, including support for the use of Oxbrya; ongoing and planned studies and related protocols, activities and expectations; regulatory submissions, review and approval to potentially expand the approved use of Oxbrya for more patients in the U.S. and to treat patients in Europe and other territories; altering the treatment, course and care of SCD and mitigating related complications; 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 payer 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 collaborations and license 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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