New Data Supporting the Potential Use of Oxbryta® (voxelotor) in Children Ages 4 to 11 Years with Sickle Cell Disease Presented at European Hematology Association 2021 Virtual Congress

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Analysis from Phase 2a HOPE-KIDS 1 Study Featured in Oral Presentation

Two Real-world Data Studies Demonstrated Sustained Improvements in Hemoglobin with Oxbryta Consistent with Results from the Phase 3 HOPE Study

SOUTH SAN FRANCISCO, Calif., June 11, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced new data from the Phase 2a HOPE-KIDS 1 Study that showed children with sickle cell disease (SCD) ages 4 to 11 years treated with Oxbryta® (voxelotor) tablets experienced significant improvements in hemoglobin levels. These data, along with results from two real-world evidence studies of Oxbryta, will be presented at the European Hematology Association (EHA) 2021 Virtual Congress, taking place online June 9-17, 2021.

“The encouraging results seen with Oxbryta treatment in younger patients living with sickle cell disease support our belief that Oxbryta can have a positive impact across a wide age range of people living with this devastating, lifelong disease by reducing the sickling and destruction of red blood cells, thereby improving anemia and hemolysis – hallmarks of the condition," said Ted W. Love, M.D., president and chief executive officer of GBT. “Additionally, we are pleased by the growing body of real-world evidence demonstrating the meaningful benefits of Oxbryta in patients ages 12 and older. Collectively, these data reinforce our commitment to seek broad access for patients and add to our confidence in the potential of this innovative medicine to address the urgent needs of sickle cell disease patients in the U.S., Europe, the Middle East and beyond.”

New Data Analysis from Phase 2a HOPE-KIDS 1 Study (Oral Abstract #S260)
A new analysis of data from 45 children with SCD ages 4 to 11 years enrolled in the open-label Phase 2a HOPE-KIDS 1 Study (GBT440-007) showed that treatment with Oxbryta (1,500 mg or weight-based equivalent dispersed in a pediatric-appropriate formulation) resulted in rapid and sustained improvements in hemoglobin. An increase in hemoglobin of greater than 1 g/dL from baseline was observed in 47% of patients as early as two weeks and sustained through 24 weeks, consistent with results in patients ages 12 years and older in the Phase 3 HOPE Study. Concurrent improvements in markers of hemolysis were also observed.

“The potentially life-threatening complications of sickle cell disease are the cumulative result of damage caused by the sickling and destruction of red blood cells that begins at a very young age. While the goal is to treat sickle cell disease early in life to prevent potential long-term consequences, current therapeutic options for younger patients are limited and do not adequately address the underlying cause of this devastating disease,” said Clark Brown, M.D., Ph.D., director of sickle cell clinical research at the Aflac Cancer and Blood Disorders Center of Children’s Healthcare of Atlanta, a primary investigator of the study. “With consistent results to published data in adults, the data from the HOPE-KIDS 1 Study underscore the potential for Oxbryta to provide important benefits in children as young as 4 years old, with a favorable safety profile.”

The findings support the equivalency of weight-based dosing in children ages 4 to 11 years and the 1,500 mg dose of Oxbryta in adolescents ages 12 to 17 years. The mean hemoglobin occupancy was 26% in children ages 4 to 11, which is consistent with results in the Phase 3 HOPE Study. In the HOPE-KIDS 1 study, Oxbryta was well tolerated and no new adverse safety signals were detected. The most commonly reported treatment-related adverse events were transient and self-limiting (diarrhea (11%), vomiting (11%) and rash (11%)).

Real-World Experience with Oxbryta
Findings from two new analyses of real-world experience with Oxbryta demonstrated improvements in hemoglobin consistent with results observed in the Phase 3 HOPE Study, which were previously published in The New England Journal of Medicine.

- An analysis of real-world outcomes in 77 patients with SCD treated in a single-center case series (Abstract #EP1209) showed that hemoglobin levels increased by an average of 2 g/dL after treatment with Oxbryta along with corresponding improvements in markers of hemolysis. A hemoglobin response greater than 1 g/dL with Oxbryta was observed in 62% of patients without the use of hydroxyurea and 87% of patients treated with hydroxyurea and Oxbryta.

The robust hematologic response was associated with improved clinical status, as measured by the Clinical Global Impression of Change (CGI-C) and Patient Global Impression of Change (PGI-C) scales, validated outcomes measures that provide holistic assessments of the effect of treatment by the physicians and patients, respectively. Few adverse events were recorded, and all were resolved with dose modification. The safety profile observed in this analysis was consistent with the approved label for Oxbryta.

- A separate study (Abstract #EP1206) provides a study design overview of RETRO, GBT’s multicenter retrospective data collection and analysis registry, which will collect real-world outcomes in up to 300 adults and adolescents with SCD treated with Oxbryta at 10 sites across the United States. The study will incorporate medical records for each patient one year before and up to one year after starting Oxbryta and will capture clinical outcome measures, health resource utilization data, and laboratory measures. An initial analysis of outcomes in 20 patients enrolled in the RETRO study at a
single study site was reported. After 12 months of treatment with Oxbryta, 50% of patients had increased hemoglobin greater than 1 g/dL.

Patient registration and data collection in the RETRO Study is ongoing. GBT also recently initiated the prospective PROSPECT Study, which is designed to enroll up to 750 patients at approximately 25 U.S. sites. GBT intends to collect data from these registries to enable deeper understanding of the long-term efficacy and safety of Oxbryta.

**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.³⁵ 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.⁴⁷

**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, 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.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.

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).

Full Prescribing Information for Oxbryta is available at [Oxbryta.com](http://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) 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](http://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 and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbryta; GBT’s commitment to seek broad patient access to Oxbryta; significance of data to be presented at the EHA Congress, including support for the use of Oxbryta and manner of dosing in children; 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 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 that 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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