



GBT Announces Five Data Presentations on Sickle Cell Disease at Upcoming EHA2022 Congress

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New real-world evidence data on Oxbryta® (voxelotor) from multicenter RETRO Study and new Phase 1 data on GBT601 to be highlighted

SOUTH SAN FRANCISCO, Calif., May 12, 2022 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced five abstracts related to its sickle cell disease (SCD) programs will be presented at the [European Hematology Association \(EHA\) 2022 Hybrid Congress](#). Presentations include new real-world evidence data on Oxbryta® (voxelotor) as well as an oral presentation of additional data from the Phase 1 study of GBT021601 (GBT601), the company's next generation sickle hemoglobin (HbS) polymerization inhibitor. In addition, initial findings from the Sickle Cell Health Awareness, Perspectives and Experiences (SHAPE) survey, a global burden of disease study assessing healthcare professional (HCP), patient and caregiver perspectives on the unmet needs in SCD, will be presented at the Congress, which will be held from June 9-12 in Vienna, Austria and online.

"Our data presentations at EHA2022 support the sustained use of Oxbryta for the treatment of hemolytic anemia in sickle cell disease, and the promise of our pipeline to improve clinical outcomes for people living with this devastating condition. Real-world evidence continues to provide valuable insights into what happens every day in clinical practice and supports important decisions regarding care," said Kim Smith-Whitley, M.D., executive vice president and head of research and development at GBT. "Data presented at this year's Congress will contribute to the growing body of evidence that could transform the treatment of sickle cell disease."

Following are highlights of GBT's presentations at the Congress.

Data from a large retrospective study of 216 patients in the United States treated with Oxbryta in a real-world setting further supports its utility in treating patients with SCD:

- New data from the multicenter **Retrospective Study to Evaluate Outcomes in Patients with Sickle Cell Disease Treated with Oxbryta (RETRO)** provide further support that treatment with Oxbryta is associated with increased hemoglobin (Hb) levels and decreased hemolytic markers. These data are consistent with those from the Phase 3 HOPE Study that led to the approval of Oxbryta.

Data from the Phase 1 studies of GBT601, which has shown promise in the clinic, supports its further development:

- Multiple daily doses of GBT601 were well tolerated in both healthy volunteers and adult SCD patients. In SCD patients, the 100 mg maintenance dose studied resulted in a mean Hb occupancy of more than 30%, increased Hb levels, reduced markers of hemolysis, and improved red blood cell (RBC) health (including improved deformability and less sickling). Additional data will be included with the oral presentation.

Data presented from two abstracts add greater insight into the disease burden and effect of Hb levels on people with SCD:

- The multinational **Sickle Cell Health Awareness, Perspectives and Experiences Survey (SHAPE)** assessed HCP perspectives on treating SCD and the patient burden of the disease. Results of the SHAPE survey underscore the negative impact of SCD on quality of life and highlight health inequities in SCD and the need for improved awareness, education and treatments that fully address end-organ damage in SCD.
- A retrospective analysis of 12 years of data from the Clinical Practice Research Datalink and the Hospital Episode Statistics databases in the UK was sufficient to observe end-organ damage events in patients with SCD. It showed that an increase in Hb of 1 g/dL was associated with a significant reduction in the risk for stroke, pulmonary hypertension, chronic kidney disease, end-stage renal disease, leg ulcers, and pneumonia, supporting the use of therapeutics that increase Hb levels in patients with SCD to protect against organ damage.

A presentation on GBT's pivotal Phase 3 studies of inclacumab, the company's P-selectin inhibitor:

- Currently in progress and enrolling patients, the two pivotal Phase 3 THRIVE studies are evaluating the safety and efficacy of inclacumab in reducing vaso-occlusive crises (VOCs) and readmissions due to VOCs. An additional THRIVE open-label expansion (OLE) study will examine the long-term safety of inclacumab in individuals with SCD.

All abstracts are now available at www.ehaweb.org. Details of the GBT presentations, which will be available in the poster hall and via the virtual event platform, are as follows:

Oral Presentation: Sickle Cell Disease

Abstract # S268: Safety, Tolerability, and Pharmacokinetic/Pharmacodynamic Results from Phase 1 Studies of GBT021601, a Next-Generation HbS Polymerization Inhibitor for Treatment of Sickle Cell Disease

Presenter: Clark Brown, M.D., Ph.D., Afac Cancer and Blood Disorders Center of Children's Healthcare of Atlanta and Department of Pediatrics, Emory School of Medicine, Atlanta, GA, USA
Sunday, June 12, 2022, 11:30 -12:45 CEST

Poster Session: Sickle Cell Disease

Abstract # P1487: Sickle Cell Health Awareness, Perspectives and Experiences (SHAPE) Survey: Findings on the Burden of Sickle Cell Disease on Patients and Their Unmet Needs as Reported by Healthcare Professionals

Presenter: Baba Inusa, M.D., Department of Paediatric Haematology, Guy's and St Thomas' Hospital, London, UK
Friday, June 10, 2022, 16:30 - 17:45 CEST

Abstract # P1486: Trials in Progress - The THRIVE Studies Evaluating the Efficacy, Safety, and Long-Term Treatment with Inclacumab, a P-Selectin Inhibitor, in Patients with Sickle Cell Disease

Presenter: Biree Andemariam, M.D., New England Sickle Cell Institute, University of Connecticut Health, Farmington, CT, USA
Friday, June 10, 2022, 16:30 - 17:45 CEST

Abstract # P1485: A Multicenter, Retrospective Study on Real-World Experience of Patients with Sickle Cell Disease Treated with Voxelotor

Presenter: Biree Andemariam, M.D., New England Sickle Cell Institute, University of Connecticut Health, Farmington, CT, USA
Friday, June 10, 2022, 16:30 - 17:45 CEST

Abstract # P1488: Association Between Hemoglobin Levels and End-Organ Damage in Sickle Cell Disease: A Retrospective Linked Primary and Secondary Care Database Analysis in England

Presenter: Paul Telfer, D.M., F.R.C.P., Queen Mary, University of London, UK
Friday, June 10, 2022, 16:30 - 17:45 CEST

About Sickle Cell Disease

Sickle cell disease (SCD) affects more than 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.³ It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.⁴ 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.^{4,5,6} 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 delivery throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.^{5,6,7,8} Complications of SCD begin in early childhood and can include neurocognitive impairment, acute chest syndrome, and silent and overt stroke, among other serious issues.⁹

About Oxbryta® (voxelotor)

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 leading to hemolysis and hemolytic anemia, 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.

In November 2019, the U.S. Food and Drug Administration (FDA) granted accelerated approval for Oxbryta tablets for the treatment of SCD in adults and children 12 years of age and older, and in December 2021, the FDA expanded the approved use of Oxbryta for the treatment of SCD in patients 4 years of age and older in the United States.¹⁰ As a condition of accelerated approval for patients ages 4 and older in the United States, 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 14 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 received the prestigious 2021 Prix Galien USA award for "Best Biotechnology Product" from The Galien Foundation.

Oxbryta has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), Oxbryta was designated by the European Commission (EC) as an orphan medicinal product for the treatment of patients with SCD, and Oxbryta was granted Promising Innovative Medicine (PIM) designation in the United Kingdom from the Medicines and Healthcare products Regulatory Agency (MHRA). In February 2022, the European Commission (EC) granted Marketing Authorization for Oxbryta for the treatment of hemolytic anemia due to SCD in adult and pediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide (hydroxyurea). In addition, the Ministry of Health and Prevention (MOHAP) in the United Arab Emirates (UAE) has granted marketing authorization for Oxbryta for the treatment of SCD in adults and children 12 years of age and older.

Please click here for [Important Safety Information](#) and full [Prescribing Information](#) including [Patient Information](#) for Oxbryta in the U.S.

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, starting with sickle cell disease (SCD). Founded in 2011, GBT is delivering on its goal to transform the treatment and care of SCD, a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta® (voxelotor), the first FDA-approved medicine that directly inhibits sickle hemoglobin (HbS) 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 Phase 3 development to address pain crises associated with the disease, and GBT021601 (GBT601), the company's next generation HbS polymerization inhibitor. 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

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, commitment, dedication, focus, goals, mission, vision, and positioning; the safety, efficacy, and mechanism of action of Oxbryta, and other product characteristics; the commercialization, awareness, delivery, availability, use, and commercial and medical potential of Oxbryta, including the use, significance and potential of related initiatives; presentation of data at EHA and their significance, including with respect to the use of Oxbryta, the potential of GBT’s pipeline, the further development of GBT601, and the use of therapeutics that increase Hb levels; the significance and use of real-world evidence; the impact of this year’s Congress, including in contributing to evidence to transform the treatment of SCD; ongoing and planned studies, clinical trials and registries, and related protocols, activities, timing, and other expectations; impacting the treatment, care, and course of SCD and mitigating related complications; safety, efficacy, mechanism of action, advancement and potential of GBT’s drug candidates and pipeline; 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 research, development, manufacture, distribution, and commercialization activities; government and third-party payer actions, including those relating to reimbursement and pricing; risks and uncertainties relating to competitive treatments 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 collaboration, license and distribution agreements; along with those risks set forth in GBT’s Annual Report on Form 10-K for the fiscal year ended December 31, 2021, 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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Contact Information:

Steven Immergut (media)
+1 650-410-3258
simmergut@gbt.com

Courtney Roberts (investors)
+1 650-351-7881
croberts@gbt.com



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