GBT Announces Upcoming Data Presentations During Virtual Edition of 25th Annual European Hematology Association Congress

May 14, 2020

Data include retrospective analysis of landmark STOP 2 Study highlighting correlation between higher hemoglobin levels and lower transcranial Doppler (TCD) flow velocities in children with sickle cell disease

SOUTH SAN FRANCISCO, Calif., May 14, 2020 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that four abstracts will be presented during the virtual edition of the 25th Annual European Hematology Association (EHA) Congress taking place June 11-14. The abstracts include a retrospective analysis of data from the landmark STOP 2 study linking higher hemoglobin levels to lower transcranial Doppler (TCD) flow velocity, a predictor of stroke risk in children with sickle cell disease (SCD), and three encore presentations of the pivotal Phase 3 HOPE Study of Oxbryta® (voxelotor). Oxbryta is the first drug approved by the U.S. Food and Drug Administration that directly inhibits sickle hemoglobin polymerization, the process that causes red blood cells to sickle and break down.

“Children with SCD have a greatly elevated risk of stroke, and the STOP 2 study provided critical insights that have informed current stroke prevention strategies. We are pleased to present data from this landmark study that further support the relationship between increased hemoglobin levels and decreased risk of stroke, as assessed by TCD flow velocity,” said Ted W. Love, M.D., president and chief executive officer of GBT. “These are important findings that support the potential for Oxbryta to have a clinically meaningful effect on stroke risk, which we are further evaluating in our HOPE-KIDS 2 post-approval confirmatory trial.”

The EHA abstracts are now available at www.ehaweb.org. Details of GBT’s poster presentations are as follows:

Friday, June 12

Session: Sickle Cell Disease
Abstract #EP1533: Correlation Between Hemoglobin Levels and Transcranial Doppler Velocities: A Retrospective STOP 2 Analysis in Children with Sickle Cell Disease
Presenter: Jenifer Voeks, Ph.D., Medical University of South Carolina
Time: 8:30 a.m. CEST

Session: Sickle Cell Disease
Abstract #EP1540: Correlation of Voxelotor Exposure with Hemoglobin Response and Measures of Hemolysis in Patients from the HOPE Study
Presenter: Jo Howard, MB BChir, MRCP, FRCPath, Guy’s and St. Thomas’ NHS Foundation Trust and King’s College London
Time: 8:30 a.m. CEST

Session: Sickle Cell Disease
Abstract #EP1534: Concomitant Hydroxyurea and Voxelotor: Results from the HOPE Study
Presenter: Russell Ware, M.D., Ph.D., Cincinnati Children’s Hospital Medical Center
Time: 8:30 a.m. CEST

Session: Sickle Cell Disease
Abstract #EP1526: Incidence of Vaso-occlusive Crisis Does Not Increase with Achieving Higher Hemoglobin Levels on Voxelotor Treatment or After Discontinuation: Analyses of the HOPE Study
Presenter: Elliott Vichinsky, M.D., UCSF Benioff Children’s Hospital Oakland
Time: 8:30 a.m. CEST

About Sickle Cell Disease
Sickle cell disease (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.[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.[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.[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]

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, GBT believes Oxbryta blocks polymerization and the resultant sickling and destruction of red blood cells, which are primary pathologies faced by every single person living with SCD. With the potential to improve hemolytic anemia and oxygen delivery, 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.[7] 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 voxelotor in its Priority Medicines (PRIME) program, and the European Commission (EC) has designated voxelotor as an orphan medicinal product for the treatment of patients with SCD.

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 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, an underlying cause of 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.

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, focus, goals and vision; the safety, efficacy and mechanism of action of Oxbryta and other product characteristics; the commercialization, delivery, availability, use, and commercial and medical potential of Oxbryta; ongoing and planned studies of Oxbryta and related protocols, activities and expectations; the significance of abstracts to be presented; transforming the treatment and care of SCD; the potential of inclacumab; and advancing GBT's pipeline, 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 has only recently established its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbryta; government and third-party payor 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 the funding and other obligations under the Pharmakon loan; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, 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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