



## **GBT Announces Upcoming Data Presentations at 24th European Hematology Association (EHA) Congress Supporting Voxelotor Sickle Cell Disease Program**

May 16, 2019

*Phase 3 HOPE Study 24-Week Efficacy Results from Full Patient Cohort to be Presented During Presidential Symposium*

*New Drug Application (NDA) Submission Planned for Voxelotor to Include HOPE Study Data Being Presented at EHA 2019*

*Company to Host Investor Webcast on Friday, June 14*

SOUTH SAN FRANCISCO, Calif., May 16, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that data supporting the company's voxelotor program in sickle cell disease (SCD) will be presented during the 24<sup>th</sup> European Hematology Association (EHA) Congress, which is taking place June 13-16 in Amsterdam.

Three abstracts have been accepted for presentation, including new 24-week efficacy and safety data from approximately 270 patients in the Phase 3 HOPE Study, which will be presented during the Presidential Symposium on Friday, June 14.

"We are pleased that the new long-term efficacy and safety data from our HOPE Study was selected for presentation during the Presidential Symposium, which showcases the top six abstracts selected by the EHA Scientific Program Committee each year. The HOPE Study results we will present at EHA represent the data we will include in our New Drug Application for voxelotor, which we are preparing to submit in the second half of this year," said Ted W. Love, M.D., president and chief executive officer of GBT. "Together with our two poster presentations, the HOPE Study data supports the potential of voxelotor to safely sustain improvements in hemoglobin levels, reduce hemolysis and be a disease-modifying treatment that could reduce the morbidity and mortality of patients with SCD."

The HOPE Study data that will be presented at the Congress includes additional efficacy and safety data not available at the time the abstracts were submitted and, as such, remains under embargo until Friday, June 14.

The EHA abstracts are now available at [www.ehaweb.org](http://www.ehaweb.org). Details of GBT's presentations are as follows:

### **Friday, June 14**

Oral Session: Presidential Symposium

Abstract #S147: Results from the Randomized Placebo-Controlled Phase 3 Hope Trial of Voxelotor in Adults and Adolescents with Sickle Cell Disease

Presenter: Jo Howard, MB BChir, MRCP, FRCPath, Guy's and St. Thomas' NHS Foundation Trust and King's College, London, UK

Time: 4:15-4:30 p.m. CEST / 10:15-10:30 a.m. ET

Location: Hall 5

Poster Session: Sickle Cell Disease

Abstract #PF740: Cerebral Blood Flow in Adolescents with Sickle Cell Anemia Receiving Voxelotor

Presenter: Jeremie H. Estépp, M.D., Assistant Member, Faculty, Hematology Department, St. Jude Children's Research Hospital, Memphis, Tenn.

Time: 5:30-7:00 p.m. CEST / 11:30 a.m.-1:00 p.m. ET

Location: Poster Area

### **Saturday, June 15**

Poster Session: Sickle Cell Disease

Abstract #PS1522: Central Physiologic Mechanisms Which Augment Oxygen Release (Bohr Effect and 2,3-DPG Binding) Are Preserved in the Presence of Voxelotor at the Therapeutic Target of 30% Hb Modification

Presenter: Mira Pochron, Ph.D., Scientist II, GBT, South San Francisco, Calif.

Time: 5:30-7:00 p.m. CEST / 11:30 a.m.-1:00 p.m. ET

Location: Poster Area

### **Investor Webcast Details**

GBT will host an investor event webcast on Friday, June 14, 2019, at 7:00 p.m. CEST / 1:00 p.m. ET to review the HOPE Study data being presented at EHA 2019. The event will be webcast live and available for replay from GBT's website at [www.gbt.com](http://www.gbt.com) in the [Investors](#) section.

### **About Sickle Cell Disease**

SCD is a lifelong inherited blood disorder caused by a genetic mutation in the beta-chain of hemoglobin, which leads to the formation of abnormal hemoglobin known as sickle hemoglobin (HbS). In its deoxygenated state, HbS has a propensity to polymerize, or bind together, forming long, rigid

rods within a red blood cell (RBC). The polymer rods deform RBCs to assume a sickled shape and to become inflexible, which causes hemolytic anemia (low hemoglobin due to RBC destruction) that can lead to multi-organ damage and early death. This sickling process also causes blockage in capillaries and small blood vessels. Beginning in childhood, SCD patients typically suffer unpredictable and recurrent episodes or crises of severe pain due to blocked blood flow to organs, which often lead to psychosocial and physical disabilities.

#### **About Voxelotor in Sickle Cell Disease**

Voxelotor (previously called GBT440) is being developed as an oral, once-daily therapy for patients with SCD. Voxelotor works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes voxelotor blocks polymerization and the resultant sickling and destruction of red blood cells. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that voxelotor may potentially modify the course of SCD. In recognition of the critical need for new SCD treatments, the U.S. Food and Drug Administration (FDA) has granted voxelotor 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.

GBT is evaluating voxelotor in the HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study, a Phase 3 clinical study in patients age 12 and older with SCD. Additionally, voxelotor is being studied in the ongoing Phase 2a HOPE-KIDS 1 Study, an open-label, single- and multiple-dose study in pediatric patients (age 4 to 17) with SCD. The HOPE-KIDS 1 Study is assessing the safety, tolerability, pharmacokinetics and exploratory treatment effect of voxelotor.

#### **About GBT**

GBT is a clinical-stage biopharmaceutical company determined to discover, develop and deliver innovative treatments that provide hope to underserved patient communities. GBT is developing its lead product candidate, voxelotor, as an oral, once-daily therapy for sickle cell disease. To learn more, please visit [www.gbt.com](http://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 about GBT's anticipated public offering, anticipated use of proceeds and other statements containing the words "anticipate," "planned," "believe," "forecast," "estimated," "expected," and "intend," among others. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements we make 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. We intend these forward-looking statements, including statements regarding our plan to submit an NDA for voxelotor under an accelerated regulatory approval pathway, the availability of, and sufficiency of our data to support, accelerated regulatory approval, the therapeutic potential and safety profile of voxelotor, including the potential to safely sustain improvements in hemoglobin levels, reduce hemolysis and be a disease-modifying treatment for SCD that could reduce morbidity and mortality, our ability to implement and complete our clinical development plans for voxelotor, our ability to engage in continued discussions with the FDA and the outcome of our discussions with the FDA, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including data from patients enrolled in our Phase 3 HOPE Study, and data from our ongoing Phase 2a HOPE-KIDS 1 Study), our plan to include the HOPE Study data presented at EHA in our NDA, regulatory review and actions relating to voxelotor, and the timing of these events, 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 are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. We 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 our control including, without limitation, the risks that our clinical and preclinical development activities may be delayed or terminated for a variety of reasons, that results of clinical trials may be subject to differing interpretations, that regulatory authorities may disagree with our clinical development plans or require additional studies or data to support further clinical investigation of our product candidates, that drug-related adverse events may be observed in clinical development, and that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2019, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.*

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