GBT Announces New Phase 2a Voxelotor Data in Adolescents with Sickle Cell Disease (SCD) at 23rd European Hematology Association (EHA) Congress

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Data from Four Additional Posters to be Presented, Including New Pharmacokinetic Data from HOPE-KIDS 1 Study Supporting Use of Weight-based Dosing Strategy for Voxelotor Clinical Trials in Children

SOUTH SAN FRANCISCO, Calif., June 15, 2018 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ:GBT) today announced new 24-week data from patients treated with the 900 mg dose of voxelotor in the ongoing HOPE-KIDS 1 Study, a Phase 2a open-label study in adolescents ages 6 to 17 years with sickle cell disease (SCD). Results demonstrated sustained and durable improvements in hemoglobin levels and a reduction in clinical measures of hemolysis with voxelotor in adolescents with SCD. The data will be presented today at the 23rd European Hematology Association (EHA) Congress in Stockholm.

"We continue to be encouraged by the results of the ongoing HOPE-KIDS 1 study, which are consistent with inhibition of HbS polymerization by voxelotor and support its ongoing clinical evaluation as a potential disease-modifying therapy for both adults and adolescents with SCD," said Ted W. Love, M.D., president and chief executive officer of GBT. "Results to date support our ongoing development of voxelotor in a broad range of patients, including in our Phase 3 HOPE Study, which is also evaluating voxelotor at doses of 900 mg and 1500 mg per day in adolescents and adults. We continue to expect to announce top-line clinical data from Part A of the HOPE Study by the end of this quarter."

Results from a Phase 2a Study (GBT440-007) Evaluating Adolescents with Sickle Cell Disease Treated with Multiple Doses of Voxelotor (GBT440), a HbS Polymerization Inhibitor (abstract #PF709)
The HOPE-KIDS 1 Study (GBT440-007), an open-label, single- and multiple-dose study, is evaluating the safety, tolerability, pharmacokinetics and exploratory treatment effect of voxelotor in a pediatric population (ages 6 to 17) with SCD. Part A of the study evaluated a single 600 mg dose of voxelotor while Part B is exploring the safety of voxelotor at doses of 900 mg and 1500 mg per day administered to patients ages 12 to 17.

The EHA presentation analyzed 22 patients who received voxelotor 900 mg/day for 24 weeks in Part B. Data showed the following results achieved with voxelotor treatment:

- Increased hemoglobin levels and improved clinical measures of hemolysis at 24 weeks, as evaluated by changes from baseline in hemoglobin, percent of reticulocytes, and percent of unconjugated bilirubin.
- Specifically, 43 percent of patients (9 of 21) achieved a hemoglobin response >1 g/dL at 24 weeks with a median hemoglobin change from baseline of 0.7 g/dL.
- Reduced daily symptoms at 24 weeks as assessed by total symptom scores (TSS), which improved in 13 of 21 patients.
- 55 percent of patients (11 of 20) had a numerical decrease in transcranial doppler (TCD) flow at 24 weeks; among hemoglobin responders (>1 g/dL), 88 percent (7 of 8) had a numerical decrease in TCD at 24 weeks.
- Favorable tolerability profile in adolescents, consistent with results in adults. Drug-related adverse events related to voxelotor were grade 1 or 2, except one grade 3 urticaria that did not recur with continued dosing. The most common drug-related adverse events (occurring in two or more patients) were nausea (12 percent), vomiting (8 percent), headache (8 percent) and rash (8 percent). There were no drug-related discontinuations due to adverse events.

Pharmacokinetics (PK) of Voxelotor (GBT440) Using Population Pharmacokinetic (PPK) and Physiologically Based Pharmacokinetic (PBPK) Modeling in Pediatric Subjects with Sickle Cell Disease (abstract #PF713)
A separate poster will be presented today at EHA summarizing the single-dose pharmacokinetics (PK) of voxelotor in children and the multiple-dose PK of voxelotor in adolescents. The analysis was based on data from approximately 6 children (ages 6 to 11) who have completed Part A of the HOPE-KIDS 1 study and 25 adolescents (ages 12 to 17) who have completed Part B.

Results showed that voxelotor PK exposures in children following a single oral 600 mg dose were higher than in adolescents or adults, suggesting that lower doses (on a weight or surface area basis) should be evaluated for future clinical trials in children. Additionally, voxelotor PK exposures in adolescents following multiple 900 mg doses were similar to those observed in adults, suggesting similar doses may be administered in adolescents. Further, physiologically-based PK modeling confirmed the need for lower doses in children younger than age 12.

Three additional posters will be presented at EHA that support GBT's voxelotor SCD program, including one on the development, translation and cultural adaptation of GBT's Sickle Cell Disease Severity Measure (SCDSM). This nine-item electronic patient reported outcome (PRO) instrument was designed by GBT with guidance from the U.S. Food and Drug Administration (FDA) to evaluate changes in SCD symptom burden in the Phase 3 **H**emoglobin **O**xygen Affinity Modulation to Inhibit Sickle Hemoglobin **P**olym**E**rization (HOPE) Study. Two encore presentations – one on the novel design of the HOPE Study, and another on findings from seven adult SCD patients with severe anemia and multiple co-morbidities who received

voxelotor through compassionate access – also will be presented.

About Sickle Cell Disease (SCD)
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 can cause blockage in capillaries and small blood vessels. Beginning in childhood, SCD patients 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. This blocked blood flow, combined with hemolytic anemia (the destruction of RBCs), can eventually lead to multi-organ damage and early death.

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 of red blood cells. With the potential to restore normal hemoglobin function and improve 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 currently 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 6 to 17) with SCD. HOPE-KIDS 1 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 and follow the company on Twitter @GBT_news.

Forward-Looking Statements
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 the therapeutic potential and safety profile of voxelotor including in pediatric and adolescent patients, our ability to implement and complete our clinical development plans for voxelotor, our ability to generate and report data from our ongoing and potential future studies of voxelotor (including our ongoing Phase 3 HOPE Study and our ongoing Phase 2a HOPE-KIDS 1 Study), 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, 2017, and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, 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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