GBT Receives FDA Breakthrough Therapy Designation for Voxelotor for Treatment of Sickle Cell Disease (SCD)

January 9, 2018

Voxelotor is First Investigational Treatment for SCD to Receive Breakthrough Therapy Designation

SOUTH SAN FRANCISCO, Calif., Jan. 09, 2018 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ:GBT) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) to voxelotor (previously called GBT440) for the treatment of sickle cell disease (SCD). Voxelotor is being developed as a disease-modifying therapy for SCD and previously received European Medicines Agency (EMA) Priority Medicines (PRIME) designation for the treatment of SCD.

"The FDA's decision to grant voxelotor the first Breakthrough Therapy designation for the treatment of sickle cell disease reflects a recognition of the promising efficacy and safety data we have collected to date for this investigational drug, as well as an acknowledgement of the overwhelming need for major advances over available therapies in the treatment of SCD patients," said Ted W. Love, president and chief executive officer of GBT. "This designation is another significant milestone for GBT as we work to expedite the development of voxelotor."

The FDA selectively grants BTD to expedite the development and review of drugs that have demonstrated preliminary clinical evidence indicating the potential for substantial improvement over available therapy. The BTD decision for voxelotor was based on clinical data submitted from the following studies:

- Preliminary efficacy and safety data from Part A of the Phase 3 HOPE Study (GBT440-031)
- Phase 1/2 study and open-label extension in adults (GBT440-001/024)
- Ongoing Phase 2 HOPE-KIDS 1 study in children age 6 to 17 (GBT440-007)
- Compassionate Access experience in adults with severe SCD (not eligible for the HOPE Study)

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 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 (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) 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 (previously called GBT440), 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, 2016, and in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, 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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