GBT Announces Publication of Data from Phase 1/2 Study of Voxelotor in Patients with Sickle Cell Disease

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—Company Plans to Submit NDA for Voxelotor Under FDA Accelerated Approval Pathway Later This Year—

SOUTH SAN FRANCISCO, Calif. , Jan. 29, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that data from its Phase 1/2 study of voxelotor in patients with sickle cell disease (SCD) were published online in *Blood*, a peer-reviewed publication of the American Society of Hematology. Voxelotor is being developed as a potentially disease-modifying therapy for SCD. GBT intends to submit a New Drug Application (NDA) for voxelotor under an accelerated approval pathway to the U.S. Food and Drug Administration (FDA) later this year.

"The findings from the Phase 1/2 study and the open-label extension played an important role in our development of voxelotor. We believe voxelotor has the potential to become a standard-of-care therapy in SCD, as evidenced by its ability to reduce anemia, hemolysis and sickling," said Ted W. Love, M.D., president and chief executive officer of GBT. "Additionally, I'm pleased that we have reached agreement with the FDA on both the accelerated approval pathway for voxelotor and for transcranial doppler (TCD) flow velocity as an acceptable primary endpoint in a post-approval confirmatory study to demonstrate stroke risk reduction. We plan on initiating our TCD study later this year. I look forward to our upcoming pre-NDA meeting with the FDA for voxelotor, which will primarily focus on the format of our planned NDA filing in the second-half of this year."

As reported in the *Blood* article, "A phase 1/2 ascending dose study and open-label extension study of voxelotor in patients with sickle cell disease," the safety, tolerability, pharmacokinetic and pharmacodynamic properties of voxelotor were demonstrated in patients with SCD. Thirty-eight patients with SCD received voxelotor at doses of 500, 700 or 1000 mg per day or placebo for 28 days; 16 patients received voxelotor at doses of 700 or 900 mg per day or placebo for 90 days. Four patients from the 90-day cohort were subsequently enrolled in an extension study and treated with a once-daily 900 mg dose of voxelotor for six months. Patients treated with voxelotor for ≥90 days showed a 1 g/dL increase in hemoglobin and a substantial and durable reduction in hemolysis and sickled red blood cells. Low hemoglobin (anemia) and chronic hemolysis (red blood cell destruction) are powerful predictors of chronic organ damage, including stroke, silent cerebral infarction, renal failure and pulmonary hypertension, as well as early mortality in SCD. Voxelotor was well tolerated with no treatment-related serious adverse events and no evidence of tissue hypoxia.

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 recently completed its evaluation of 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 Global Blood Therapeutics
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 two therapies for the potential treatment of sickle cell disease, including its late-stage product candidate, voxelotor, as an oral, once-daily therapy. 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 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 the availability of, and sufficiency of our data to support, accelerated regulatory approval, our plan to submit an NDA for voxelotor under an accelerated approval pathway, the therapeutic potential and safety profile of voxelotor, including the potential for voxelotor to be a disease-modifying treatment for SCD and to become a standard-of-care therapy in SCD, the potential for TCD flow velocity to serve as an acceptable primary endpoint in a confirmatory study to demonstrate stroke risk reduction, our plan to initiate a TCD study, our plan for a pre-NDA meeting, 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 additional data from patients enrolled in our ongoing Phase 3 HOPE Study, and data in 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 in our Quarterly Report on Form 10-Q for the quarter ended September 30, 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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