GBT Announces U.S. Food and Drug Administration Acceptance of New Drug Application and Priority Review for Voxelotor for the Treatment of Sickle Cell Disease

September 5, 2019

Priority Review Shortens FDA Review Time to Six Months from Standard 10 Months

NDA Supported by Data from Phase 3 HOPE Study, which Demonstrated Statistically Significant and Sustained Improvements in Hemoglobin with Voxelotor

SOUTH SAN FRANCISCO, Calif., Sept. 05, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the U.S. Food and Drug Administration (FDA) has accepted for filing the company's New Drug Application (NDA) seeking accelerated approval for voxelotor, an oral, once-daily therapy in development for the treatment of sickle cell disease (SCD). If approved, voxelotor would be the first therapy available to patients that targets hemoglobin polymerization, the root cause of SCD damage.

The FDA granted Priority Review for the NDA for voxelotor, which provides for a six-month review, and assigned a Prescription Drug User Fee Act (PDUFA) target action date of February 26, 2020. The Agency also indicated in the NDA filing acceptance notification letter that it is not currently planning to hold an advisory committee meeting to discuss the application for voxelotor.

"The FDA's acceptance of our NDA for voxelotor under Priority Review is a major milestone in the development of this investigational therapy and further illustrates the significance the Agency places on getting important and innovative treatments to individuals living with SCD as quickly as possible," said Ted W. Love, M.D., president and chief executive officer of GBT. "We look forward to working with the FDA during this process, with the goal of potentially changing the treatment paradigm for SCD."

Priority Review is granted to therapies that the FDA determines have the potential to provide a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious condition. Under PDUFA, a Priority Review targets a review time of six months compared to a standard review time of 10 months.

The NDA for voxelotor is supported by data from the multi-national Phase 3 HOPE Study of voxelotor in patients ages 12 and older with SCD who enrolled in the study from 60 institutions across 12 countries. The HOPE Study results were recently published in *The New England Journal of Medicine*. Voxelotor has been granted FDA Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD.

About Accelerated Approval
The FDA grants accelerated approval under subpart H for new drugs that address serious or life-threatening illnesses and appear to provide meaningful therapeutic benefit to patients over existing treatments on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. Drugs approved under subpart H are subject to preapproval of promotional materials, are required to be further evaluated in at least one post-marketing study to confirm clinical benefit and are subject to withdrawal if post-marketing studies fail to confirm such benefit.

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, 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 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 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 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 development plans for voxelotor and the potential benefits of voxelotor for SCD patients 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, the therapeutic potential and safety profile of voxelotor, including the potential to be a disease-modifying therapy for SCD, the potential for voxelotor to be approved and to become a new standard of care for treating adolescents and adults with SCD, 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 data from patients enrolled in our Phase 3 HOPE Study, and data from our ongoing Phase 2a HOPE-KIDS 1 Study), regulatory review and actions relating to voxelotor, our potential commercial launch of 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 June 30, 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.

Contact Information:
Stephanie Yao (investors & media)
GBT
650-741-7730
investors@gbt.com

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Source: Global Blood Therapeutics, Inc.