GBT Announces Upcoming Presentations at National Sickle Cell Disease Association of America (SCDAA) Convention

October 4, 2018

SOUTH SAN FRANCISCO, Calif., Oct. 04, 2018 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that three abstracts related to its sickle cell disease (SCD) development programs, including voxelotor, have been accepted for oral presentation during the 46th Annual National Sickle Cell Disease Association of America (SCDAA) Convention in Baltimore, MD, taking place October 10-13, 2018. Additionally, GBT will host a lunch symposium at the conference titled, “Hemolytic Anemia in Sickle Cell Disease: What is It and Why Does it Matter?” at 12:00 p.m. ET on Thursday, October 11. The symposium will focus on helping attendees understand what hemolytic anemia is and how it contributes to complications over the lifetime of a sickle cell patient, such as chronic organ damage. This will be followed by open dialogue with attendees to understand how patients perceive the importance of disease modification and organ protection in considering the impact of new treatment options as they emerge.

Details of the oral presentations are as follows:

Friday October 12

Abstract:	Finding SCD Patients In Michigan—A Case Study
Presenter:	Wanda Whitten-Shurney, MD, Sickle Cell Disease Association of America – Michigan Chapter; Jeanne M. Loboda, GBT
Oral Session:	Community Based Research
Time:	3:30-3:45 p.m. ET
Location:	Baltimore/Annapolis

Abstract:	Compassionate-Use Voxelotor (GBT440) For Up To 2 Years In Patients With Severe Sickle Cell Disease And Life-Threatening Comorbidities
Presenter:	Kenneth R. Bridges, MD, GBT
Oral Session:	Clinical, Basic Science and Translational Research
Time:	3:30-3:45 p.m. ET
Location:	Constellation C

Abstract:	Digital Engagement In The Sickle Cell Community
Presenter:	Robin C. Howard, GBT
Oral Session:	Community Based Research
Time:	4:15-4:30 p.m. ET
Location:	Baltimore/Annapolis

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 (the destruction of RBCs) 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 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 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 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
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, 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 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 our Quarterly Report on Form 10-Q for the quarter ended June 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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Global Blood Therapeutics, Inc.