Global Blood Therapeutics Announces New Details on its Novel Patient Reported Outcomes (PRO) Tool to be used in Phase 3 HOPE Study

December 5, 2016

-- PRO designed to establish clinical benefit –

-- First time a PRO to be used as a secondary clinical endpoint in a SCD registrational study --

--Company to Host Webcast Today at 12:15 p.m. PT to Discuss ASH Data Presentations —

SOUTH SAN FRANCISCO, Calif., Dec. 05, 2016 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ:GBT) today announced that the methodology used to develop the Patient Reported Outcome (PRO) tool that will be used as a secondary clinical endpoint in the company's planned pivotal Phase 3 clinical trial of GBT440 in people with sickle cell disease (SCD), titled the HOPE (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) Study, was presented at the 58th American Society of Hematology (ASH) Annual Meeting & Exposition (abstract #4760). The nine-item Sickle Cell Disease Severity Measure (SCDSM) has been designed by GBT to assess the entire range of SCD symptoms, to distinguish between good days and bad days based on symptoms and to measure all crisis events regardless of health care utilization.

"Previous SCD studies have focused on vaso-occlusive crisis (VOC) defined as a painful crisis requiring hospital or emergency room utilization as the traditional clinical endpoint. However, studies have shown that VOC is not a full measure of the burden of SCD daily symptoms because many episodes of pain are managed at home rather than in a doctor's office, hospital or emergency room and are thus under-reported. VOC is the tip of the iceberg," said Wally Smith, M.D., Florence Neal Cooper Smith Professor of Sickle Cell Disease Director, and Director of the comprehensive sickle cell program at Virginia Commonwealth University. "By having this new electronic patient reported outcomes (PRO) tool, we will be able to more reliably assess daily symptom severity, with a single total symptom score, potentially allowing us to accelerate the development of urgently needed new SCD therapies."

PRO development used qualitative and quantitative analyses and was undertaken in collaboration with SCD patient groups and external experts, and with active engagement with the Clinical Outcomes Assessment group of the United States Food and Drug Administration (FDA). The instrument was designed with a specific set of nine questions to produce a score to measure the full range of daily SCD severity in adult and adolescent SCD patients. The HOPE PRO includes questions specific to pain, energy level and fatigue, concentration and breathlessness- the symptoms that matters the most to patients with SCD. When qualified by following FDA guidance, a PRO instrument can be used as a clinical endpoint to establish benefit for regulatory approval. In the HOPE Study, the HOPE PRO instrument will be administered on a hand-held electronic device and will explore the clinical benefit of GBT440 in SCD. It is designed to distinguish between good days and bad days based on symptoms, which should be sensitive to all pain crises, with or without healthcare utilization. The PRO will also be able to assess an improvement in symptoms from baseline, such as improved fatigue. During Part A of the HOPE Study, the PRO instrument will quantify the magnitude of changes in daily total symptom scores over three months. The HOPE PRO complies with the FDA's issued guidance for PRO development.

"We are excited to have developed, in conjunction with the SCD community, the first PRO for SCD suitable for use as a clinical endpoint that could support potential FDA approval of a drug for the treatment of SCD. The HOPE PRO is designed to be a reliable, valid and sensitive measurement that meets regulatory requirements as a clinical outcome assessment tool, is easy for patients to use, and will generate scores that are clinically meaningful," said Ted W. Love, M.D., president and chief executive officer of GBT. "With the utilization of the HOPE PRO, our highly innovative HOPE Study trial design will allow us to measure the core symptoms of SCD that matter most to patients - pain and fatigue - as well as other key SCD symptoms."

Also presented at ASH yesterday were data from the ongoing Phase 1/2 GBT440-001 study that further support the safety and efficacy profile of GBT440 as a potentially disease-modifying therapy for sickle cell disease (SCD) and results showing how GBT440 is metabolized in healthy subjects.

Investor Event Webcast Details
Today, Monday, December 5, at 12:15 p.m. PT, members of GBT's management team and Jo Howard, MB BChir, MRCP, FRCPath, of Guy's and St Thomas' NHS Foundation Trust, Wally R. Smith, MD of Virginia Commonwealth University, and Jeremy Hobart, BSc PhD FRCP Dip Public Health, of Peninsula Schools of Medicine and Dentistry will review GBT440 ASH data presentations. The event will be webcast live and will be available for replay from the Investors section of GBT's website at www.globalbloodtx.com for 30 days.

About GBT440 in Sickle Cell Disease
GBT440 is being developed as an oral, once-daily therapy for patients with SCD. GBT440 works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes GBT440 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 GBT440 may dramatically 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 GBT440 both fast track and

orphan drug designations and the European Commission (EC) has designated GBT440 as an orphan medicinal product for the treatment of patients with SCD. In addition to the ongoing Phase 1/2 GBT440-001 trial, GBT440 is being evaluated in an open-label, single and multiple dose study in adolescents (age 12 to 17) with SCD. This study is assessing the safety, tolerability, pharmacokinetics and exploratory treatment effect of GBT440.

Additionally, GBT440 will be evaluated in the pivotal Phase 3 HOPE Study. This randomized, double-blind, placebo-controlled, multi-national trial will enroll up to 400 patients age 12 and older with SCD who have had at least one episode of vaso-occlusive crisis (VOC) in the previous year. The study will be conducted in two parts: Part A will compare GBT440 administered at doses of 900 or 1,500 mg per day vs. placebo in up to 150 patients treated for at least 12 weeks, and Part B will include 250 patients randomized to placebo or a dose of GBT440 based on Part A. The main objectives of Part A are to select the optimal dose, define the final secondary endpoints for Part B, and qualify the PRO instrument. The primary efficacy endpoint of the HOPE Study is the proportion of patients who achieve a greater than 1 g/dL increase in hemoglobin at 24 weeks of treatment compared with baseline. Key secondary efficacy endpoints include the effect of GBT440 on SCD symptom exacerbation (as measured by the HOPE PRO instrument), overall SCD symptoms, traditionally defined VOCs, hospitalizations and red blood cell transfusions.

About Sickle Cell Disease (SCD)
SCD is a lifelong inherited blood disorder caused by a genetic mutation in the beta-chain of hemoglobin, leading to formation of abnormal hemoglobin known as sickle hemoglobin, or 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 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 Global Blood Therapeutics
Global Blood Therapeutics, Inc. is a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need. GBT is developing its lead product candidate, GBT440, as an oral, once-daily therapy for sickle cell disease and will initiate its Phase 3 clinical trial by the end of 2016. GBT is also investigating GBT440 for the treatment of hypoxemic pulmonary disorders in two ongoing Phase 2a studies in patients with idiopathic pulmonary fibrosis. To learn more, please visit: www.globalbloodtx.com.

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 GBT440, our ability to implement our clinical development plans for GBT440, the timing of, and our ability to generate data from, our ongoing Phase 1/2 clinical trial of GBT440 and our ability to begin and generate data from our HOPE Study, and regarding the use of the PRO in our HOPE Study, 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 regulatory authorities may disagree with our clinical development plans or require additional studies or data to support further clinical investigation of our product candidates, and that drug-related adverse events may be observed in later stages of clinical development, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, and in our Quarterly Reports on Form 10-Q for the quarters ended March 31, 2016, June 30, 2016 and September 30, 2016, 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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