Global Blood Therapeutics Announces Pivotal Study for GBT440 in Sickle Cell Disease with Primary Hemoglobin Endpoint

October 24, 2016

HOPE – A Phase 3 Clinical Trial to Initiate in December

Company to Host Conference Call and Webcast Today at 1:30 p.m. PT/ 4:30 p.m. ET

SOUTH SAN FRANCISCO, Calif., Oct. 24, 2016 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ:GBT), a biopharmaceutical company developing novel therapeutics for the treatment of grievous blood-based disorders with significant unmet need, today announced that it has reached agreement with the U. S. Food and Drug Administration (FDA) regarding the design of its pivotal trial for GBT440 in adults and adolescents with sickle cell disease (SCD).

The Phase 3 HOPE (**H**emoglobin **O**xygen Affinity Modulation to Inhibit HbS **P**olym**E**rization) Study will be conducted at leading SCD sites globally and will enroll adults and adolescents with SCD who have had at least one episode of vaso-occlusive crisis (VOC) in the previous year. The HOPE Study is expected to begin screening patients by December with top-line data anticipated in the first half of 2019.

"We are extremely pleased with our interactions and outcome of our pre-Phase 3 process with the FDA. We believe that this agreed-upon study design underscores our common goal to address the urgent unmet medical need for new disease-modifying preventative therapies for SCD and the importance of innovative regulatory strategies to bring new therapies to market to treat these patients," said Ted W. Love, M.D., president and chief executive officer of GBT. "Solidification of the path forward for our pivotal study is an important milestone and we believe the HOPE Study design has a strong grounding in GBT440's mechanism of action, including its potential to fundamentally modify the course of the disease by inhibiting sickle hemoglobin polymerization."

HOPE Study Design
The HOPE Study is a randomized, double-blind, placebo-controlled, multi-national, Phase 3 trial, which 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 HOPE Study will be conducted in two-parts:

Part A - Compare two dose levels of GBT440 – 900 mg and 1500 mg versus placebo. Part A will include up to 150 patients.

Part B - To 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 Patient Reported Outcome (PRO) instrument.

The primary efficacy endpoint of the HOPE Study will be the proportion of patients who achieve a >1 g/dL increase in hemoglobin at 24 weeks of treatment vs baseline. Our discussions with the FDA have focused on a pathway to full approval based on the HOPE Study, by meeting the primary and at least one key secondary endpoint.

Key secondary efficacy endpoints will include the effect of GBT440 on SCD symptom exacerbation, which will be measured by our PRO instrument, in addition to overall SCD symptoms as compared to placebo. The PRO is administered on a hand-held electronic device, and is designed to capture the full range of daily SCD symptoms. We will also assess traditionally defined VOCs as well as hospitalizations and red blood cell transfusions as secondary endpoints.

"Previous SCD studies have generally focused on VOC, defined as a painful crisis requiring hospital or emergency room utilization. But we know that patients have 4-5 times more frequent painful crises, with or without utilization. As a result, the burden of painful crises is dramatically under-reported," said Wally Smith, M.D., Florence Neal Cooper Smith Professor of Sickle Cell Disease Director, a comprehensive sickle cell program at Virginia Commonwealth University. "By utilizing the PRO, the innovative design of the HOPE Study should allow measurement of the true burden of SCD painful crises and other symptoms."

Conference Call with Management
Management will host a conference call to provide a program update today at 1:30 p.m. PT/ 4:30 p.m. ET. To participate in the conference call, please dial (844) 471-0808 (domestic) or (480) 696-7309 (international) and refer to conference ID 3206614. Live audio of the conference call will be simultaneously webcast and will be available under the Investors and Media section of the company's website at www.globalbloodtx.com.

The webcast will be archived under the investors and media section of the company's website and will be available for replay for at least one month after the conference call.

About GBT440 in Sickle Cell Disease
GBT440 is being developed as an oral, once-daily therapy for patients with sickle cell disease (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 be capable of modifying the progression of SCD.

The U.S. Food and Drug Administration (FDA) has granted GBT440 both fast track and orphan drug designations for the treatment of patients with SCD, in recognition of the critical need for new treatments. GBT440 is currently being evaluated in an ongoing Phase 1/2 clinical study. This randomized, placebo-controlled, double-blind, single and multiple ascending dose study is evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of GBT440 in both healthy subjects and adults with SCD. Additionally, in adolescents (age 12 to 17) with SCD, an open-label, single and multiple dose study, evaluating the safety, tolerability, pharmacokinetics and exploratory treatment effect of GBT440 is ongoing.

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. In its lead clinical program, GBT is developing GBT440 as an oral, once-daily therapy for sickle cell disease (SCD) and is currently evaluating GBT440 in a Phase 1/2 study in both healthy subjects and adults with SCD and a Phase 2a study in adolescents with SCD. GBT is also investigating GBT440 for the treatment of hypoxemic pulmonary disorders in an ongoing Phase 2a study 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 the initiation of, and availability of top-line data from, our HOPE Study, our ability to enroll patients in the study, and the scope and number of endpoints required to be met to support regulatory approval, 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 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 or regulatory approval of our product candidates, and that drug-related or other 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 Report on Form 10-Q for the quarter ended June 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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