Global Blood Therapeutics Announces First Cohort Dosed in Phase I/II Trial of GBT440 in Sickle Cell Disease

Company Expects Study to Provide Early Clinical Proof of Concept (POC) for Disease-Modifying Treatment in Second Half of 2015

SOUTH SAN FRANCISCO, CA – January 12, 2015 – Global Blood Therapeutics (GBT), a biopharmaceutical company developing novel, small molecule therapeutics for the treatment of severe blood disorders, today announced that the first cohort of eight subjects has been dosed in the company's Phase I/II clinical trial of its lead drug candidate, GBT440, for the treatment of sickle cell disease (SCD).

GBT440 is an oral, once daily dosing, direct-acting sickle hemoglobin (HbS) modifier for the chronic, prophylactic treatment of SCD. The drug works by increasing hemoglobin's affinity for oxygen. Since oxygenated hemoglobin does not polymerize, GBT440 blocks polymerization of HbS and the resultant sickling of red blood cells (RBCs). With the promise of restoring normal hemoglobin function, GBT440 may be capable of arresting the progression of SCD.

"The initiation of this first clinical trial of GBT440 is a critical milestone," said Ted W. Love, M.D., chief executive officer of GBT. "The study has the potential to demonstrate early clinical proof of concept as soon as the second half of 2015."

The Phase I/II clinical study is a randomized, placebo-controlled, double-blind, single and multiple ascending dose study of the safety, tolerability, pharmacokinetics and pharmacodynamics of GBT440 in healthy subjects and patients with SCD. The trial will evaluate the anti-sickling and clinical benefit of GBT440 in SCD patients. Approximately 128 subjects will be enrolled and randomized 6:2 to receive daily oral dosing of GBT440 or placebo for one day (single dose) and up to 28 days (multiple doses).

GBT presented new data for GBT440 at the 2014 American Society of Hematology (ASH) Conference last month in San Francisco. Findings reported in both oral and poster presentations highlighted the drug's ability to prevent sickling of RBCs, inhibit the polymerization of deoxygenated HbS, and restore normal RBC function in preclinical SCD models. In addition, in a transgenic sickle cell mouse model, repeat oral dosing showed prolonged red blood cell half-life and decreased reticulocyte counts. These results demonstrate GBT440's potential to disrupt fundamental SCD pathophysiology and, in turn, halt progression of the disease.

About Sickle Cell Disease (SCD)

Sickle cell disease (SCD) includes a group of inherited disorders caused by a genetic mutation leading to formation of sickle hemoglobin (HbS). When deoxygenated, HbS molecules aggregate

into long polymers, the primary event in the molecular pathogenesis of SCD. Polymerized hemoglobin chains distort normally flexible RBCs into rigid, sickled cells.

Sickled RBCs impair blood flow, causing hemolysis (breakdown of red blood cells) and vascular occlusion. As a consequence, patients with SCD suffer acute and chronic complications including unpredictable and recurrent episodes of severe pain, progressive organ damage, stroke and a shortened life expectancy.

About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company developing novel, small molecule therapeutics to treat grievous blood disorders. The company is addressing serious, non-malignant blood-based conditions for which there are currently no effective cures and only limited therapy. Lead drug candidate, GBT440, is a potentially disease-modifying therapeutic for patients with sickle cell disease. GBT440 is in a Phase I/II clinical trial. In addition to GBT440, the company is advancing pipeline research programs addressing hereditary angioedema (HAE) and hypoxic cardiopulmonary disorders.

To learn more, please visit: www.globalbloodtx.com.

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