



Global Blood Therapeutics' GBT440 Demonstrates Ability to Positively Impact Fundamental Sickle Cell Disease (SCD) Processes in Studies Presented at 2014 American Society of Hematology (ASH) Conference

Groundbreaking Chronic Prophylactic Treatment for SCD is Focus of Oral and Poster Presentations, Data Paves Way for Advancing GBT440 into Clinical Studies

SOUTH SAN FRANCISCO, CA – December 8, 2014 – Global Blood Therapeutics (GBT), a biopharmaceutical company developing novel, orally available therapeutics for the treatment of severe chronic blood diseases, announced today it has presented promising data from multiple preclinical studies of GBT440, its lead drug candidate, at the 56th ASH Annual Meeting and Exposition. Data were presented today in an oral presentation and on Saturday, December 6th in a poster presentation. Positive GBT440 study results highlight the drug's ability to prevent sickling of red blood cells (RBCs), inhibit the polymerization of deoxygenated HbS, improve RBC deformability and reduce whole blood viscosity in preclinical models of sickle cell disease (SCD). In addition, repeat oral dosing in sickle cell mice showed prolonged red blood cell half-life and decreased reticulocyte counts when 30-40% Hb occupancy was achieved. By disrupting the fundamental disease processes, GBT440 is positioned to potentially stop the progression of SCD. The ASH conference is currently being held (December 6-9, 2014) in San Francisco.

GBT is developing GBT440 (formerly known as GTx011) in a Phase I/II trial that is currently recruiting participants.

Details of the oral presentation (ASH Abstract #217) are as follows:

This morning, David Archer, Ph.D., Associate Professor, Department of Pediatrics, Emory University School of Medicine/Aflac Cancer and Blood Disorders Center delivered an oral presentation entitled, "***GTx011, a Potent Allosteric Modifier of Hemoglobin Oxygen Affinity, Prevents RBC Sickling in Whole Blood and Prolongs RBC Half-Life in Vivo in a Murine Model of Sickle Cell Disease,***" reporting the following findings:

- Single oral doses of GBT440 demonstrate increased Hb-oxygen affinity and ex-vivo anti-sickling activity
- Repeat oral doses of GBT440 also demonstrate increased Hb-oxygen affinity and ex-vivo anti-sickling activity. Additionally, repeat oral doses prolonged RBC half-life and decreased reticulocyte counts in mice where 30-40% target Hb occupancy was achieved

“Based on the study findings presented today, I believe that GBT440 shows promise as a disease-modifying treatment for the chronic management of patients with sickle cell disease,” stated Dr. Archer.

Details of the poster presentation (Abstract #1370) are as follows:

On Saturday, December 6, 2014, a poster presentation entitled, “*GTx011, an Anti-Sickling Compound, Improves SS Blood Rheology By Reduction of HbS polymerization Via Allosteric Modulation of O₂ Affinity,*” reported that GBT440 has been shown to:

- Allosterically affect the heme pocket of hemoglobin
- Induce a delay in polymerization and reduce cytoplasmic hyperviscosity in a dose dependent manner
- Improve SS RBC deformability under hypoxic conditions

“We believe that GBT440 has the potential to completely change the treatment paradigm for SCD and the promising data presented at the ASH conference supports that belief,” stated Ted W. Love, M.D., chief executive officer of Global Blood Therapeutics. “GBT440 interrupts the disease process at its root cause. Based on the drug’s targeted mechanism of action, structural and binding characteristics, compelling preclinical safety and predicted once-daily oral dosing, we believe it has the potential to be the best-in class drug candidate for the chronic prophylactic treatment of SCD.”

About Sickle Cell Disease (SCD)

Sickle cell disease includes a group of inherited blood disorders that affect over 100,000 Americans and millions of individuals worldwide. SCD is characterized by the production of abnormal hemoglobin, known as hemoglobin S (HbS). HbS polymerization turns normally flexible, rounded red blood cells into rigid, sickle-shaped cells which have a significantly shorter lifespan and greatly impaired capillary flow and oxygen delivery. As a consequence of this vascular occlusion, patients with SCD suffer acute and chronic complications including unpredictable and recurrent episodes of severe pain, progressive organ damage, stroke and limited life expectancy.

About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company developing novel, orally available small molecule therapeutics for the treatment of severe chronic blood diseases. The company is addressing severe, non-malignant blood-based diseases for which there are currently no effective cures and only limited therapeutic options. Lead drug candidate, GBT440, is a potentially best-in-class disease-modifying therapeutic for patients with sickle cell disease. Preclinical studies have shown promising results and the company expects to initiate clinical testing in January 2015. In addition to GBT440, the company is advancing pipeline research

programs addressing additional important diseases of the blood, including hereditary angioedema (HAE).

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

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