



Global Blood Therapeutics to Present New Data Highlighting Therapeutic Potential of GBT440 in Sickle Cell Disease (SCD) at 2014 American Society of Hematology (ASH) Conference

Groundbreaking Disease-Modifying Treatment for SCD is Focus of Oral and Poster Presentations

SOUTH SAN FRANCISCO, CA – December 2, 2014 – Global Blood Therapeutics (GBT), a biopharmaceutical company developing novel, orally available therapeutics for the treatment of severe chronic blood diseases, announced today that data from multiple preclinical studies of GBT440, its lead drug candidate, will be presented in both oral and poster sessions at the 56th ASH Annual Meeting and Exposition. The conference is being held December 6-9, 2014 in San Francisco.

GBT is developing GBT440 (formerly known as GTx011) as a potentially best-in-class disease-modifying therapy for sickle cell disease (SCD). By blocking polymerization of sickle hemoglobin (HbS) and the resultant sickling of red blood cells, GBT440 is designed to transform treatment by addressing the disease's underlying cause, not just its symptoms. With the promise of restoring normal hemoglobin dynamics, GBT440 is positioned as potentially the first therapeutic capable of preventing and halting the progression of SCD.

Data from several *in vitro* studies and an *in vivo* mouse model of sickle cell disease demonstrate the ability of GBT440 to block the fundamental processes of HbS polymerization and red blood cell sickling.

Details of the oral presentation are as follows:

Abstract #217: 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

Presenter: David Archer, Ph.D.

Session: 113: Hemoglobinopathies, Excluding Thalassemia - Basic and Translational Science: Innovative Therapeutic Approaches in Sickle Cell Disease

Date/Time: Monday, December 8, 2014, 7:00 a.m. PST

Location: Yerba Buena Ballroom Salon 8 (San Francisco Marriott Marquis)

Details of the poster presentation are as follows:

Abstract #1370: GTx011, an Anti-Sickling Compound, Improves SS Blood Rheology By Reduction of HbS polymerization Via Allosteric Modulation of O₂ Affinity

Session: 113. Hemoglobinopathies, Excluding Thalassemia – Basic and Translational Science: Poster I

Date/Time: Saturday, December 6, 2014, 5:30 – 7:30 p.m. PST

Location: West Building, Level 1 (Moscone Center)

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.

Contact Information:

Stephanie Diaz (investors)
Vida Strategic Partners
415-675-7401
sdiaz@vidasp.com

Tim Brons (media)
Vida Strategic Partners
415-675-7402
tbrons@vidasp.com