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Global Blood Therapeutics Presents New Preclinical Data on Lead Sickle Cell Disease Drug Candidate, GTx011, at American Society of Hematology Annual Meeting

-- GTx011 Undermines Mechanism of Sickle Cell Disease and Prevents Sickling of Red Blood Cells --

SOUTH SAN FRANCISCO, Calif. – December 9, 2013 – Global Blood Therapeutics, a biopharmaceutical company focused on developing novel, orally-available therapeutics for chronic blood diseases, announced new preclinical data demonstrating that GTx011, its lead drug candidate for the treatment of sickle cell disease (SCD), prevents the sickling of red blood cells in preclinical models of SCD. The data were presented today during an oral presentation titled GTx011, a Potent Allosteric Modifier of Hemoglobin Oxygen Affinity, Delays Polymerization and Prevents Sickling (abstract number 316) at the 55th American Society of Hematology (ASH) Annual Meeting and Exposition in New Orleans. Additional data on the effects of GTx011 were presented at ASH on Sunday, December 8 in a poster presentation titled GTx011, a Novel Agent That Improves Rheological Properties of Sickle Cell Blood By Increasing Oxygen Affinity For Hemoglobin (abstract number 2207).

“This is our first report that GTx011, our lead product candidate, significantly delays in vitro polymerization of the mutated form of hemoglobin that causes SCD, and also markedly reduces sickling of red blood cells from preclinical models of disease and from SCD patients. With these properties, GTx011 addresses the underlying cause of this devastating blood disease, which may lead to benefits in both the acute symptoms and chronic consequences of SCD,” said Mark A. Goldsmith, M.D., Ph.D., chief executive officer of Global Blood Therapeutics. “These data, including preclinical pharmacokinetics, help to validate our best-in-class hemoglobin modifier strategy, and enable us to continue advancing toward clinical development.”

SCD is an inherited disorder caused by a single genetic mutation leading to the production of hemoglobin S (HbS), an altered form of hemoglobin, the vital oxygen-transport protein found in red blood cells. In SCD, polymerization of deoxygenated HbS causes the sickling of red blood cells, which in turn causes impaired blood flow, organ damage and reduced life expectancy. Global Blood Therapeutics researchers designed and synthesized a novel series of compounds to overcome these properties of HbS and with attractive characteristics to support further development.

Key findings reported included:

• GTx011 increases the affinity of HbS for oxygen, and is the most potent compound currently reported to be in development for SCD.
• GTx011 delays HbS polymerization in a dose-dependent manner. The profile of GTx011 is similar to that of natural hemoglobins that also delay polymerization and are known to ameliorate disease in individuals with one or two copies of the gene for HbS.
• GTx011 prevents the sickling of SCD red blood cells under prolonged low-oxygen conditions in a dose-dependent manner.
• GTx011 reverses the sickling of red blood cells in a dose-dependent manner.
• GTx011 reduces the viscosity of blood from SCD patients.
• GTx011 exhibits high oral bioavailability and consistent exposure in multiple animal species.
• GTx011 demonstrates consistent pharmacokinetic and anti-sickling activity in an animal model of SCD.

These data indicate that GTx011 has the potential to be a novel, oral, chronic prophylactic therapeutic to treat the underlying cause of SCD.

About Sickle Cell Disease
Sickle Cell Disease (SCD) is a global health challenge with very limited treatment options and no therapies that address the underlying cause of the disease. SCD is caused by a single genetic mutation that alters the oxygen-transport protein hemoglobin and results in the “sickling” – or change to a crescent shape – of red blood cells. There are approximately 100,000 SCD patients in the United States and more than 7 million worldwide. SCD patients suffer severe pain, vaso-occlusion, anemia, organ damage, high risk of stroke and renal insufficiency. There is typically an early onset of symptoms and reduced life expectancy. While blood transfusions and bone marrow transplants can be used in select settings, these procedures are costly, require matching donors and extensive medical infrastructure for delivery, and themselves can lead to life-threatening complications – factors limiting their accessibility to patients in developing countries. Hydroxyurea is the only approved pharmaceutical treatment in the United States.

About Global Blood Therapeutics
Global Blood Therapeutics is a biopharmaceutical company developing novel, orally-available therapeutics for 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. Global Blood Therapeutics is focused on the critical need for therapeutics that address the underlying causes of blood diseases, not just the symptoms. The company’s extensive drug discovery capabilities — combining world-class medicinal chemistry with deep expertise in blood biology — are driving its product pipeline of mechanism-based therapeutics, including programs in sickle cell disease and hereditary angioedema. Global Blood Therapeutics is a private company launched in 2012 by Third Rock Ventures with an experienced leadership team and renowned scientific founders. For more information, please visit www.globalbloodtx.com.

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