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Third Rock Ventures Launches Global Blood Therapeutics with \$40.7 Million to Revolutionize the Treatment of Chronic Blood-Based Diseases

*Company Developing Novel, Oral Treatments for Severe Genetic Disorders;
Marks First Company Formation & Launch out of Third Rock Ventures' West Coast Office*

San Francisco and Boston – June 14, 2012 – [Third Rock Ventures, LLC](#) today announced the formation of [Global Blood Therapeutics, Inc.](#) and its \$40.7 million Series A financing of the company. The company's unique SHAPE Platform is enabling the discovery and development of oral, small molecule drugs that change the shapes of key blood proteins to modify their functions, enhance vital biological pathways and drive therapeutic benefits. Global Blood Therapeutics is building a pipeline of innovative medicines for severe, genetic blood diseases for which there are currently no effective cures and only extremely limited therapeutic options. Founders of the company include Charles Homcy, M.D., and Craig Muir of Third Rock Ventures, David Phillips, Ph.D., co-founder of COR Therapeutics and co-founder with Dr. Homcy of Portola Pharmaceuticals, and three leading scientific researchers at the University of California, San Francisco: Matthew Jacobson, Ph.D., professor, pharmaceutical chemistry, Andrej Sali, Ph.D., professor, bioengineering and therapeutic sciences, and Jack Taunton, Ph.D., associate professor, cellular and molecular pharmacology.

“Blood is one of the largest and most accessible organs and is a rich source of biologically and clinically validated targets,” said [Mark A. Goldsmith, M.D., Ph.D.](#), chief executive officer of Global Blood Therapeutics and venture partner at Third Rock Ventures, LLC. “Global Blood Therapeutics aims to provide game-changing treatments for serious, genetic blood diseases that are well-understood but poorly served today, by developing innovative medicines directed against clinically validated targets. Through our SHAPE Platform and our world-class team with a strong track record in science and drug discovery and development, we are positioned to develop rapidly a new generation of safe and effective oral drugs that could truly change the treatment paradigm on behalf of patients.”

Global Blood Therapeutics' lead program focuses on Sickle Cell Disease (SCD), one of the earliest rare genetic diseases to be defined on a molecular basis. It is caused by a single mutation that alters the oxygen-transport protein hemoglobin and results in the “sickling” – or change to a crescent shape – of red blood cells. With 100,000 patients in the United States and more than 15

million worldwide, SCD is a global health challenge with very limited treatment options and no therapies that address the underlying cause of the disease.

To address such worldwide need, Global Blood Therapeutics' is focused on the discovery and development of a pipeline of new therapies that change the shapes of key blood proteins to treat chronic blood-based diseases and genetic disorders. Specifically, the [SHAPE Platform](#) brings together the most advanced computational biology and protein-ligand modeling with medicinal chemistry and empiric screening capabilities focused on allosteric modulation, while leveraging the Global Blood Therapeutics team's leading expertise in blood biology and track record of success in drug discovery and development. This approach will enable Global Blood Therapeutics in the development of orally available compounds that are potent, have a rapid onset of action and ultimately should translate into high therapeutic efficacy and decreased toxicity.

“Our vision at Third Rock Ventures is to improve patients' lives decisively through innovation, and the unique approach and stellar team at Global Blood Therapeutics are positioned to revolutionize how some of the most severe, blood-based genetic diseases are treated,” said Charles Homcy, M.D., venture partner at Third Rock Ventures. “The launch of Global Blood Therapeutics, the first company fully conceived of and formed out of our West Coast office, underscores Third Rock's commitment to our goal and marks the ongoing success of our bi-coastal model and innovative, hands-on company ideation process. Global Blood expands our strong and growing portfolio of exciting companies on both coasts.”

In addition to Dr. Goldsmith, who is former chief executive officer and currently executive chairman of Constellation Pharmaceuticals, Inc., the Global Blood Therapeutics management team includes acting chief scientific officer Brian Metcalf, Ph.D., who previously served as executive vice president and chief drug discovery scientist at Incyte Corp. and senior vice president, discovery chemistry and platform technologies at SmithKline Beecham (now GlaxoSmithKline); and chief technology officer Craig Muir, partner at Third Rock Ventures, former senior vice president at Codon Devices and former vice president, platform technologies at Millennium Pharmaceuticals.

About Sickle Cell Disease (SCD)

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 100,000 SCD patients in the United States and more than 15 million worldwide. Additionally, approximately five percent of the world's population carries trait genes for hemoglobin disorders, mainly sickle cell disease and thalassemia (*Source: World Health Organization*). SCD patients suffer severe pain, vasoocclusion, anemia, organ damage, high risk of stroke and renal insufficiency. There is 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 and has only limited efficacy and multiple toxicities that limit its use.

About Global Blood Therapeutics

Global Blood Therapeutics is a product-focused company building a pipeline of innovative, oral medicines to revolutionize the treatment of chronic blood-based diseases and genetic disorders for which there are currently no available cures and only extremely limited therapeutic options. The company's SHAPE Platform enables the discovery and development of oral, small molecule drugs that change the shapes of key blood proteins to modify their functions, enhance vital biological pathways and drive therapeutic benefits. Global Blood Therapeutics' approach combines the most advanced computational biology and protein-ligand modeling with medicinal chemistry and empiric screening capabilities focused on allosteric modulation, while leveraging its team's world-leading expertise in blood biology and proven success in drug discovery and development. 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.

About Third Rock Ventures

Third Rock Ventures is a venture capital firm founded in 2007 with the mission to launch transformative life sciences companies. With more than \$800 million and two funds under management, the firm is focused on working with passionate entrepreneurs to build exceptional companies working in areas of disruptive science that will make a difference in the lives of patients. The firm has assembled a team with deep expertise and a proven track record of building respected and successful life sciences companies. With decades of complementary, cross-functional operational and leadership experience, the Third Rock team actively engages with its portfolio companies to provide hands-on strategy and experience to successfully launch companies with the best vision, science, operations, people and culture. With offices in Boston, MA and San Francisco, CA, Third Rock is well positioned geographically to closely collaborate with its portfolio companies to achieve their goals. To learn more about Third Rock and its portfolio companies, please visit www.thirdrockventures.com.

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