Global Blood Therapeutics Announces Initiation of Phase 2a Study of GBT440 in Adolescents with Sickle Cell Disease

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SOUTH SAN FRANCISCO, Calif., June 29, 2016 /PRNewswire/ -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT), a biopharmaceutical company developing novel therapeutics for the treatment of grievous blood-based disorders with significant unmet needs, today announced it has initiated a Phase 2a study of GBT440 in adolescents with sickle cell disease (SCD). GBT is developing GBT440 as a potential disease-modifying therapy for SCD.

"We are pleased to initiate our Phase 2a study of GBT440 in adolescents. The initiation of this study in adolescent SCD patients is an important milestone toward our goal of developing GBT440 for patients of all ages and genotypes," said Ted W. Love, M.D., chief executive officer of GBT. "GBT440 has the potential to be a well-tolerated, once-daily therapy to address the underlying pathology of SCD rather than just treat the symptoms. We believe it could be particularly impactful in these young patients before much of the irreversible damage associated with SCD occurs."

About the Phase 2a GBT440-007 Study
GBT440-007 is an open-label, single and multiple dose study that is evaluating the safety, tolerability, pharmacokinetics and exploratory treatment effect of GBT440 in adolescents age 12 to 17 years with SCD. The study is being conducted in two parts: in Part A, six subjects will receive a single dose of GBT440, and in Part B, 24 subjects will receive multiple doses of GBT440 for up to 28 days.

The primary objective of Part A is to characterize the pharmacokinetics of GBT440 and the primary objective of Part B is to explore the safety of multiple doses of GBT440 administered to adolescent SCD patients.

About GBT440
GBT440 is being developed as an oral, once-daily therapy for patients with sickle cell disease (SCD). GBT440 works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes GBT440 blocks polymerization and the resultant sickling of red blood cells (RBCs). With the potential to restore normal hemoglobin function and improve oxygen delivery, GBT believes that GBT440 may be capable of modifying the progression of SCD.

The U.S. Food and Drug Administration (FDA) has granted GBT440 both Fast Track and Orphan Drug designation for the treatment of patients with SCD in recognition of the critical need for new treatments. GBT440 is currently being evaluated in the ongoing Phase 1/2 GBT440-001 study. This randomized, placebo-controlled, double-blind, single and multiple ascending dose study is evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of GBT440 in both healthy subjects and patients with SCD.

About Sickle Cell Disease (SCD)
Sickle cell disease (SCD) is an inherited blood disorder caused by a genetic mutation in the beta-chain of hemoglobin, leading to formation of abnormal hemoglobin known as sickle hemoglobin, or HbS. In its deoxygenated state, HbS has a propensity to polymerize, or bind together forming long, rigid rods within a red blood cell (RBC). The polymer rods deform RBCs to assume a sickled shape and to become inflexible, which can cause blockage in small blood vessels. Beginning in childhood, SCD patients suffer unpredictable and recurrent episodes or crises of severe pain due to blocked blood flow to organs, which often lead to psychosocial and physical disabilities. This blocked blood flow, combined with hemolytic anemia (the destruction of RBCs), can eventually lead to multi-organ damage and early death.

About Global Blood Therapeutics
Global Blood Therapeutics, Inc. (GBT) is a clinical-stage biopharmaceutical company dedicated to discovering, developing and commercializing novel therapeutics to treat grievous blood-based disorders with significant unmet need. GBT is developing its lead product candidate, GBT440, as an oral, once-daily therapy for sickle cell disease (SCD) and is currently evaluating GBT440 in adults and adolescents. In addition to GBT440 for the treatment of SCD, GBT is engaged in research and development activities targeted toward hypoxemic pulmonary disorders, including idiopathic pulmonary fibrosis (IPF), and hereditary angioedema (HAE). To learn more, please visit: www.globalbloodtx.com.

Forward-Looking Statements
Statements we make in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. We intend these forward-looking statements, including statements regarding the therapeutic potential of GBT440 in adolescents with SCD and our plans regarding the enrollment of patients in our Phase 2a study of GBT440 in adolescents with SCD and the timing of these events, to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. We can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved, and furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are

beyond our control including, without limitation, the risks that our clinical and preclinical development activities may be delayed or terminated for a variety of reasons, that regulatory authorities may disagree with our clinical development plans or require additional studies or data to support further clinical investigation of our product candidate, and that drug-related adverse events may be observed in later stages of clinical development, along with those set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015 and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/global-blood-therapeutics-announces-initiation-of-phase-2a-study-of-gbt440-in-adolescents-with-sickle-cell-disease-300292235.html

SOURCE Global Blood Therapeutics, Inc.

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