Global Blood Therapeutics Announces GBT440 Granted Orphan Drug Designation in Europe for Treatment of Sickle Cell Disease

November 30, 2016

SOUTH SAN FRANCISCO, Calif., Nov. 30, 2016 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ:GBT), today announced that the European Commission (EC), acting on a positive recommendation from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), has designated GBT440 as an orphan medicinal product for the treatment of sickle cell disease (SCD).

"Receiving orphan designation from the EC marks a significant milestone both for the SCD community and for GBT," said Ted W. Love, M.D., president and chief executive officer of GBT. "SCD is a devastatingly severe disease with limited treatment options, and this designation, together with our fast track and orphan drug designations by the United States Food and Drug Administration, reflect the recognition of the broader regulatory community of this urgent unmet medical need."

European orphan designation is granted to drugs that are intended for the treatment of life-threatening or chronically debilitating rare diseases for which no therapeutic options either exist or are satisfactory. Rare diseases are those defined as having a prevalence of less than five in 10,000 in Europe. The designation provides sponsors with development and commercial incentives, including 10 years of market exclusivity, designated product specific consultation by EMA, and certain exemptions from, or reductions in, regulatory fees.

About GBT440
GBT is developing GBT440 as an oral, once-daily therapy for patients with sickle cell disease. GBT440, a hemoglobin modifier, 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, 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.

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 RBCs. 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. 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 and expects to initiate its Phase 3 clinical trial by the end of 2016. GBT is also investigating GBT440 for the treatment of hypoxemic pulmonary disorders in two ongoing Phase 2a studies in patients with idiopathic pulmonary fibrosis. 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 SCD, our ability to conduct, and to generate data from, our clinical studies, regulatory review of our product candidates 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 GBT440 may not provide the clinical benefits that we anticipate, 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 September 30, 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.

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