Global Blood Therapeutics Announces Initiation of Phase 2a Study of GBT440 in Idiopathic Pulmonary Fibrosis

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SOUTH SAN FRANCISCO, Calif., June 20, 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, a hemoglobin modifier, in adults with idiopathic pulmonary fibrosis (IPF) and hypoxemia.

Hypoxemia (abnormally low levels of oxygen in the blood) can lead to tissue hypoxia (a deficiency in the amount of oxygen reaching the tissues), which is believed to play a key role in adverse patient outcomes in IPF. GBT440 has been shown in preclinical studies to increase oxygen saturation in hypoxic conditions. Because of this ability, GBT is developing GBT440 as a potential treatment for hypoxemia, associated with acute and chronic lung disorders, such as IPF, in which the lungs cannot supply adequate oxygen to the blood.

"Current treatment for IPF is targeted at slowing lung fibrosis but there has been no improvement in oxygenation and symptom burden of the disease. Supplemental oxygen therapy, which is used to treat the hypoxemia and its associated breathlessness, has local and systemic risks and poses a significant burden to patients. For patients with this fatal disease, there is a critical need for a therapeutic drug that can improve oxygenation without those risks and improve quality of life," said Ted W. Love, M.D., chief executive officer of GBT. "Proof of concept has been established in three animal model studies suggesting that hemoglobin modification may increase oxygen saturation and improve oxygen delivery to tissues. Based on those data, we believe that GBT440 is a promising therapeutic option for IPF, and we are advancing the clinical development of this oral, once-daily compound with the initiation of this Phase 2a study."

About the Phase 2a GBT440-006 Study

GBT440-006 is a randomized, double-blind, placebo-controlled study of GBT440 that is evaluating safety, tolerability, pharmacokinetics and effect on hypoxemia in patients with IPF. A total of 18 IPF patients will be enrolled and randomized to one of two doses of GBT440 or placebo administered orally once daily for 28 days. An additional 12 patients may be enrolled following an independent safety review for a total of up to 30 patients.

In addition to safety, tolerability and pharmacokinetics of GBT440, the change from baseline in oxygen saturation both at rest and after exercise will be explored.

About IPF

Idiopathic pulmonary fibrosis (IPF) is a fatal disease characterized by irreversible, progressive scarring of the lungs. As a patient's lung scarring worsens, the lungs cannot properly move oxygen into the bloodstream and, as a result, the body's tissues do not get the oxygen they need. The cause of IPF is unknown and there is no cure. IPF inevitably causes shortness of breath and destruction of healthy lung tissue, resulting in hypoxemia, tissue hypoxia, and ultimately organ dysfunction. Patients with IPF typically experience progressive worsening of lung function over time, requiring the use of supplemental oxygen and frequent hospitalizations in the late stages of the disease.

IPF typically affects individuals over the age of 50, and the median survival after diagnosis is approximately 2 to 3 years.

About GBT440

GBT is developing GBT440 as an oral, once-daily therapy for patients with sickle cell disease (SCD) and for the potential treatment of hypoxemic pulmonary disorders, including IPF. GBT440, a hemoglobin modifier, works by increasing hemoglobin's affinity for oxygen. Emerging data suggest that hemoglobin modifiers such as GBT440 have the potential to restore normal hemoglobin function and increase oxygen uptake in the lungs, resulting in improved oxygen delivery to tissues.

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 both healthy subjects and SCD patients in a randomized, placebo-controlled, double-blind Phase 1/2 clinical trial. 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 IPF and other hypoxemic disorders and our plans regarding the enrollment of patients in our Phase 2a study of GBT440 in IPF, 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 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-idiopathic-pulmonary-fibrosis-300286943.html

SOURCE Global Blood Therapeutics, Inc.

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