



GBT Expands Sickle Cell Disease Pipeline with Exclusive In-license of Two Novel Small Molecule Programs from Sanofi S.A.

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SOUTH SAN FRANCISCO, Calif., March 16, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced it has entered into an agreement with Sanofi S.A. to exclusively in-license worldwide rights to two early-stage research programs in sickle cell disease (SCD): one that pursues a novel anti-sickling mechanism and another that leverages a new approach to reduce inflammation and oxidative stress. These mechanisms are distinct and potentially complementary to that of Oxbraya[®] (voxelotor) tablets, a novel hemoglobin S polymerization inhibitor approved in the United States for the treatment of SCD in patients ages 12 years and older. The programs, from Sanofi's Bioverativ subsidiary, supplement GBT's existing pipeline and support the company's strategy to address SCD from multiple approaches.

"We envision a future in which sickle cell disease is a well-managed condition with the potential for a functional cure in the form of patient-friendly oral therapies. As we work toward this vision and our goal to transform the treatment and care of people living with this devastating disease, we are advancing our robust internal research programs with disease-modifying potential while continually exploring partnership opportunities across a variety of mechanisms," said Jung E. Choi, chief business and strategy officer of GBT. "These novel discovery programs represent promising approaches that we believe may have the potential to lead to meaningful improvements for patients."

Under the terms of the agreement, GBT will conduct all research, development, regulatory and commercialization activities worldwide. Sanofi will receive an upfront payment and is entitled to payments up to approximately \$353 million upon achievement of development, regulatory and commercial milestones and single-digit tiered royalties on worldwide net sales.

About Sickle Cell Disease

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States,¹ an estimated 52,000 people in Europe,² and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.¹ It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.¹ SCD is a lifelong inherited rare blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.³ Due to a genetic mutation, individuals with SCD form abnormal hemoglobin known as sickle hemoglobin. Through a process called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped and rigid.³⁻⁵ The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.⁴⁻⁷

About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities. Founded in 2011, GBT is delivering on its goal to transform the treatment and care of sickle cell disease (SCD), a lifelong, devastating inherited blood disorder. The company has introduced Oxbraya[®] (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of red blood cell sickling in SCD. GBT is also advancing its pipeline program in SCD with inclacumab, a P-selectin inhibitor in development to address pain crises associated with the disease, and GBT021601 (GBT601), the company's next-generation hemoglobin S polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next wave of treatments for SCD. To learn more, please visit <https://gbt.com> and follow the company on Twitter @GBT_news.

Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995, including statements containing the words "will," "anticipates," "plans," "believes," "forecast," "estimates," "expects" and "intends," or similar expressions. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements 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. GBT intends these forward-looking statements, including statements regarding GBT's priorities, dedication, commitment, focus, goals, mission and vision; the Sanofi agreement, including rights, obligations and potential activities, results and payments thereunder; the research programs under the Sanofi agreement, including their mechanism of action and potential to complement Oxbraya, supplement GBT's pipeline, support GBT's strategy, and lead to improvements for patients; exploring partnership opportunities; safety, efficacy and mechanism of action of Oxbraya and other product characteristics; significance of reducing hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbraya; ongoing and planned studies and related protocols, activities and expectations; altering the treatment, course and care of SCD and mitigating related complications; potential and advancement of GBT's pipeline, including inclacumab and other product candidates; and working on new targets and discovering, developing and delivering treatments, 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 GBT makes this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect GBT's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the company and on assumptions the company has made. GBT 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 GBT's control, including, without limitation, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on GBT's business, including commercialization activities, regulatory efforts, research and development, corporate development activities and operating results, which will depend on future developments that are highly uncertain and cannot be accurately predicted, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease; the risks that GBT is continuing to establish its commercialization capabilities and may not be able to successfully commercialize Oxbraya; risks associated with GBT's dependence on third parties for development, manufacture, distribution and commercialization activities related to Oxbraya; government and

third-party payor actions, including those relating to reimbursement and pricing; risks and uncertainties relating to competitive products and other changes that may limit demand for Oxbryta; the risks regulatory authorities may require additional studies or data to support continued commercialization of Oxbryta; the risks that drug-related adverse events may be observed during commercialization or clinical development; data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval; compliance with obligations under the Pharmakon loan; and the timing and progress of activities under GBT's research collaborations; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2020, filed with the U.S. Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in GBT's subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, GBT assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

References

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