



GBT and Syros Partner to Discover, Develop and Commercialize Novel Therapies for Sickle Cell Disease and Beta Thalassemia

Collaboration Combines GBT's Therapeutic Area Leadership with Power of Syros' Gene Control Platform to Find New Medicines to Induce Fetal Hemoglobin

Syros to Receive \$20 Million Upfront, Three Years of Preclinical Research Funding and Milestone Payments

SOUTH SAN FRANCISCO, Calif. and CAMBRIDGE, Mass., Dec. 18, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) and Syros Pharmaceuticals, Inc. (NASDAQ: SYRS) today announced that they have entered into a collaboration to discover, develop and commercialize novel therapies for sickle cell disease (SCD) and beta thalassemia. Under the agreement, Syros will use its leading gene control platform to identify therapeutic targets and discover drugs that induce fetal hemoglobin, and GBT will receive an option to obtain an exclusive worldwide license to develop, manufacture and commercialize products resulting from the collaboration.

"The discovery and development of novel therapeutic approaches to treat sickle cell disease has been a driving force for GBT since we were founded," said Ted W. Love, M.D., president and CEO of GBT. "We believe that Syros' approach to inducing fetal hemoglobin is one of the most promising ways to identify the next generation of therapies to treat sickle cell disease and beta thalassemia at a fundamental level – upstream of serious complications such as organ damage, organ failure and early death. We will continue to seek the best scientific approaches to transform the treatment of these devastating lifelong diseases."

Using its gene control platform to elucidate mechanisms controlling gamma globin gene expression, Syros identified components of LRF (leukemia/lymphoma-related factor) and the NuRD (nucleosome remodeling and histone deacetylation) complex that could serve as potential targets to switch on the gamma globin gene, which is normally silenced a few months after birth. By turning on gamma globin expression, GBT and Syros aim to induce the production of fetal hemoglobin, which is known to exert protective effects on the red blood cells of patients with SCD and beta thalassemia and mitigate the clinical manifestation of these diseases.

"We believe it is possible to provide a functional cure for patients with sickle cell disease or beta thalassemia by switching on the gamma globin gene with an oral medicine," said Nancy Simonian, M.D., CEO of Syros. "Partnering with GBT, an established leader in sickle cell disease with proven research, development, manufacturing and commercialization capabilities, allows us to expand and accelerate our program, exploring multiple approaches in parallel with the aim of bringing much-needed new therapies to market for patients with sickle cell disease and beta thalassemia as quickly as possible."

Syros' drug discovery program in SCD was highlighted recently in an oral presentation at the 61st American Society of Hematology (ASH) Annual Meeting, as well as in an ASH press briefing. In that presentation, Syros described its discovery of a fetal hemoglobin repressor that, when knocked down in primary cells and an erythroid cell line expressing adult hemoglobin, induced fetal hemoglobin in nearly 100% of cells and increased total fetal hemoglobin levels to 40%, exceeding levels that are associated with a functional cure in SCD patients.

Terms of the Agreement

Under the terms of the agreement, GBT will pay Syros \$20 million upfront and fund up to \$40 million in preclinical research for at least three years. Should GBT exercise its option under the agreement, Syros could receive up to \$315 million in option exercise, development, regulatory, commercialization and sales-based milestones per product candidate and product resulting from the collaboration. Syros would also receive mid- to high-single digit royalties on sales of products resulting from the collaboration. In addition, Syros would have the option to co-promote the first product resulting from the collaboration in the United States.

About GBT

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 Oxbryta™ (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of 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. In addition, GBT's drug discovery teams are working on new targets to develop the next generation of treatments for SCD. To learn more, please visit www.gbt.com and follow the company on Twitter [@GBT_news](https://twitter.com/GBT_news).

About Syros Pharmaceuticals

Syros is redefining the power of small molecules to control the expression of genes. Based on its unique ability to elucidate regulatory regions of the genome, Syros aims to develop medicines that provide a profound benefit for patients with diseases that have eluded other genomics-based approaches. Syros is advancing a robust pipeline of development candidates, including SY-1425, a first-in-class oral selective RAR α agonist in a Phase 2 trial in a genomically defined subset of acute myeloid leukemia patients, and SY-5609, a highly selective and potent oral CDK7 inhibitor in investigational new drug application-enabling studies in cancer. Syros also has multiple preclinical and discovery programs in oncology and monogenic diseases, including sickle cell disease. For more information, visit www.syros.com and follow us on Twitter (@SyrosPharma) and LinkedIn.

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 the current expectations of GBT and Syros, 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 and Syros each intend these forward-looking statements, including statements regarding the ability of the parties to discover, develop and commercialize novel therapies for SCD and beta thalassemia under the collaboration, the scientific and therapeutic potential of Syros' gene control platform and approach to inducing fetal hemoglobin, the exercise by GBT of its option under the collaboration agreement, the potential milestone payments and royalties due to Syros under the collaboration agreement, and Syros' option to co-promote the first product resulting from the collaboration in the United States, 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 and Syros make this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect the current views of GBT and Syros about their respective plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the companies and on assumptions the companies have made. Neither GBT nor Syros can give any 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 the control of GBT and Syros including, without limitation, the timing and progress of, and any data generated from, the parties' research and development activities under the collaboration, and the amount and timing of resources devoted by each of the parties to activities under the collaboration, along with those risks set forth in GBT and Syros' respective Annual Reports on Form 10-K for the fiscal year ended December 31, 2018, and most recent Quarterly Reports on Form 10-Q filed with the U.S. Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in the companies' subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, neither GBT nor Syros assumes any obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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