Sangamo Therapeutics Logo

Sangamo And Bioverativ Announce FDA Acceptance Of IND Application For ST-400 -- A Gene-Edited Cell Therapy Candidate -- To Treat Beta-Thalassemia

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RICHMOND, Calif. and WALTHAM, Mass., Oct. 2, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, and Bioverativ Inc. (NASDAQ: BIVV), a global biopharmaceutical company focused on the discovery, development, and commercialization of innovative therapies for hemophilia and other rare blood disorders, announced today that the U.S. Food and Drug Administration (FDA) has accepted the Investigational New Drug (IND) application for ST-400, a gene-edited cell therapy candidate for people with transfusion-dependent beta-thalassemia. Sangamo and Bioverativ are developing ST-400 as part of an exclusive worldwide collaboration to develop and commercialize gene-edited cell therapies for beta-thalassemia and sickle cell disease.

"We are very pleased with the FDA's acceptance of the IND for ST-400 for the treatment of beta-thalassemia and look forward to initiating the first clinical trial," said Edward Conner, M.D., chief medical officer at Sangamo. "We believe the precision, efficiency and specificity of zinc finger nuclease gene editing technology will differentiate ST-400 among other genomic therapies in development for beta-thalassemia."

"Beta-thalassemia is a serious, lifelong blood disorder, and many children and adults with the disease require frequent and demanding blood transfusions that may lead to iron overload and long-term organ damage," said Tim Harris, Ph.D., D.Sc., executive vice president of research and development at Bioverativ. "The advancement of ST-400 demonstrates our commitment to progressing novel science that has the potential to make a meaningful, lasting difference in the lives of people with beta-thalassemia."

The IND enables Sangamo to initiate a Phase 1/2 clinical trial to assess the safety, tolerability and efficacy of ST-400 in adults with transfusion-dependent beta-thalassemia. Sangamo expects to open several clinical sites across the United States and begin enrolling patients in the first half of 2018.

Beta-thalassemia is an inherited blood disorder caused by mutations in the beta-globin gene that leads to reduced or absent production of adult hemoglobin, the protein in red blood cells that carries oxygen to cells throughout the body. The disorder causes the destruction of red blood cells, which results in severe anemia and reduced oxygen transport to various tissues in the body.

According to the World Health Organization, there are approximately 100,000 treated beta-thalassemia patients worldwide, with ~19,000 of those in the United States and Europe.[1] The majority of these patients are transfusion-dependent, and their current standard of care includes a chronic regimen of red blood cell transfusions, which may lead to iron overload and organ damage even with daily iron chelation therapy. Allogeneic bone marrow transplant may be a treatment option for these patients if a suitable donor can be found, but carries substantial risks such as graft-versus-host disease and chronic morbidity.

About ST-400 and the Phase 1/2 Clinical Trial
ST-400 is an autologous cell therapy that involves gene editing of a patient's own hematopoietic stem cells (HSCs) using zinc finger nuclease (ZFN) technology. It is being developed with the aim of providing a one-time treatment for people with transfusion-dependent beta-thalassemia by increasing production of fetal hemoglobin, which can more effectively carry oxygen, potentially eliminating the need for chronic blood transfusions. As part of the Phase 1/2 clinical trial protocol, a patient's HSCs are isolated from the blood, and the cells then undergo ex-vivo gene editing using ZFNs to modify a specific sequence of the BCL11A gene that suppresses fetal hemoglobin production in erythrocytes. Following a conditioning regimen, patients will be infused with their own modified HSCs, with the goal of producing increased amounts of fetal hemoglobin to compensate for the decrease in functional beta-globin levels, potentially resolving the need for chronic blood transfusions and ameliorating the complications from major organ failure that frequently arise from the disease.

About the Sangamo and Bioverativ collaboration
Sangamo and Bioverativ have an exclusive worldwide collaboration to develop and commercialize ZFN-mediated gene-edited cell therapies for the treatment of beta-thalassemia and sickle cell disease. Based on the terms of the agreement, Sangamo is responsible for conducting the ST-400 Phase 1/2 clinical trial, and Bioverativ will be responsible for subsequent worldwide clinical development, manufacturing, and commercialization.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company has open Phase 1/2 clinical trials in Hemophilia A and Hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo has an exclusive, global collaboration and license agreement with Pfizer Inc. for gene therapy programs for Hemophilia A, with Bioverativ Inc. for hemoglobinopathies, including beta-thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at www.sangamo.com.

About Bioverativ
Bioverativ is a global biopharmaceutical company dedicated to transforming the lives of people with hemophilia and other rare blood disorders through world-class research, development and commercialization of innovative therapies. Launched in 2017 following separation from Biogen Inc., Bioverativ

builds upon a strong heritage of scientific innovation and is committed to actively working with the blood disorders community. The company's mission is to create progress for patients where they need it most and its hemophilia therapies when launched represented the first major advancements in hemophilia treatment in more than two decades. For more information, visit bioverativ.com or follow @bioverativ on Twitter.

Sangamo's Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the therapeutic potential of gene editing and ST-400, including the potential of ST-400 as a one-time treatment option for people with beta-thalassemia, the planned Phase 1/2 clinical trial of ST-400, including its design and Sangamo's expectations for opening clinical sites and enrolling patients and the timing thereof, as well as other statements that are not historical facts. These forward-looking statements are based on Sangamo's current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with: gene therapy product candidate development and the inherent uncertainty of clinical success, including the risks that Sangamo and/or Bioverativ may encounter unanticipated toxicity or adverse events in, or fail to demonstrate the efficacy of ST-400 in, clinical development and that the planned Phase 1/2 clinical trial may otherwise fail to validate and support the tolerability and efficacy of ST-400; Sangamo's substantial dependence on the clinical success of its lead therapeutic programs; the initiation, enrollment and completion of the stages of its clinical trials, including Sangamo's potential inability to enroll the planned Phase 1/2 clinical trial of ST-400 in a timely manner or at all; technological challenges; the lengthy and uncertain regulatory approval process; Sangamo's and Bioverativ's ability to develop a commercially viable ST-400 product or other products under the collaboration; technological developments by competitors and others in the genomic therapy field; and Sangamo's dependence on its collaboration with Bioverativ for the development of ST-400 and its ability to maintain its collaboration with Bioverativ. A more detailed discussion of these and other risks and uncertainties may be found under the caption "Risk Factors" and elsewhere in Sangamo's SEC filings and reports, including Sangamo's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 and future filings and reports by Sangamo. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

Bioverativ Safe Harbor

This press release contains forward-looking statements, including statements about the potential benefits, safety and effects of ST-400, and expected timing and enrollment of clinical trials. These statements may be identified by words such as "believe," "expect," "may," "plan," "potential," "will" and similar expressions, and are based on Bioverativ's current beliefs and expectations. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. Factors which could cause actual results to differ materially from Bioverativ's current expectations include: uncertainties relating to the initiation, enrollment and completion of stages of clinical trials; unexpected concerns may arise from data, analysis or results obtained during clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of product candidates; risks and uncertainties relating to collaborations; Bioverativ's reliance on third parties over which it may not have control; or Bioverativ may encounter other unexpected hurdles. For more detailed information on the risks and uncertainties associated with Bioverativ's drug development and commercialization activities, please review the Risk Factors section of Bioverativ's most recent annual or quarterly report filed with the Securities and Exchange Commission. Any forward-looking statements speak only as of the date of this press release and Bioverativ assumes no obligation to update any forward-looking statements.

References

[1]*World Health Organization. Global Epidemiology of Haemoglobin Disorders and Derived Service Indicators. Available at:* http://www.who.int/bulletin/volumes/86/6/06-036673-table-T3.html*. Accessed on: September 28, 2017.*

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