



## **BIOGEN IDEC AND SANGAMO BIOSCIENCES ANNOUNCE GLOBAL COLLABORATION TO DEVELOP TREATMENTS FOR HEMOGLOBINOPATHIES**

January 9, 2014

*--Partnership to Focus on Developing Novel Therapies  
for Sickle Cell Disease and Beta-Thalassemia--*

[Biogen Idec](#) (NASDAQ:BIIB) and [Sangamo BioSciences, Inc.](#) (NASDAQ: SGMO) announced today an exclusive worldwide collaboration and license agreement focused on the development of therapeutics for hemoglobinopathies, inherited conditions that result from the abnormal structure or underproduction of hemoglobin. The agreement will enable Biogen Idec to further enhance its expertise in non-malignant hematology by leveraging Sangamo's proprietary genome-editing technology platform to develop treatments targeting sickle cell disease (SCD) and beta-thalassemia.

"Our collaboration with Sangamo is expected to help us expand our capabilities to develop treatments for people with serious, inherited hematologic conditions," said Douglas E. Williams, Ph.D., Biogen Idec's executive vice president of research and development. "Building upon emerging science related to fetal hemoglobin regulation, we intend to develop Sangamo's novel gene-editing technology to create a single approach that has the potential to functionally cure both sickle cell disease and beta-thalassemia."

Sangamo's proprietary zinc finger nuclease (ZFN) genome-editing technology enables multiple pathways to treat SCD and beta-thalassemia. The technology can be used to precisely target and knock out key regulators of gene expression, or can be used to precisely insert a new corrective gene to replace the defective copy.

"We are delighted to partner our hemoglobinopathies programs with Biogen Idec," said Edward Lanphier, Sangamo's president and chief executive officer. "Biogen Idec is a leader in drug development and has a history of successfully translating cutting edge science into treatments that provide life-changing clinical benefit for patients. This alliance is further validation of our ZFP platform as a transformative technology and accelerates our goal of developing a novel class of therapeutics which has the potential to revolutionize the treatment of genetic diseases."

Under the terms of the agreement, Sangamo is responsible for all research and development activities through the first clinical proof of concept trial in beta-thalassemia, and both companies will perform activities to enable submission of an Investigational New Drug (IND) application for SCD. Biogen Idec will be responsible for subsequent worldwide clinical development and commercialization of products arising from the alliance. Sangamo retains an option to co-promote any licensed product to treat SCD and beta-thalassemia in the United States.

Biogen Idec will provide Sangamo with an upfront payment of \$20 million and will reimburse Sangamo for its internal and external research and development program-related costs. Sangamo may also receive additional payments of approximately \$300 million based on the achievement of certain development, regulatory, commercialization and sales milestones, as well as double digit royalties on product sales.

The transaction has been approved by the boards of directors of both companies and is subject to customary closing conditions including expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 in the United States.

### **About Sangamo's ZFN Therapeutic Approach to Hemoglobinopathies**

Sangamo's proprietary ZFN genome-editing technology enables multiple approaches to the correction of SCD and beta-thalassemia. Both diseases manifest after birth, when patients switch from producing functional fetal gamma-globin to a mutant form of adult beta-globin, which results in their condition. Naturally occurring increased levels of therapeutic fetal hemoglobin have been shown to reduce the severity of both SCD and beta-thalassemia disorders in adulthood. In hematopoietic stem cells (HSCs), Sangamo's genome editing can be used to precisely disrupt key transcriptional regulators to reverse the switch from expression of the mutant adult beta-globin back to the production of functional fetal gamma-globin, or the technology can be used to precisely insert a new corrected beta-globin gene to replace the defective copy. Data from this program were recently presented at the 55th Annual Meeting of the American Society of Hematology (ASH).

A bone marrow transplant (BMT), of HSCs from a "matched" related donor (allogeneic BMT) is curative for both diseases. However, this therapy is limited due to the scarcity of matched donors and the significant risk of Graft versus Host Disease (GvHD) after transplantation of the foreign cells. By performing genome editing in HSCs that are isolated from and subsequently returned to the same patient, an autologous HSC transplant, Sangamo's approach eliminates both the need for a matched donor and the risk of acute and chronic GvHD. The ultimate goal of this approach is to develop a one-time curative treatment for SCD and beta-thalassemia.

In May 2013, Sangamo was awarded a \$6.4 million Strategic Partnership Award from the California Institute for Regenerative Medicine (CIRM) to develop this potentially curative ZFP Therapeutic for beta-thalassemia. The four-year grant provides matching funds for preclinical work that will support an IND application and a Phase 1 clinical trial in transfusion-dependent beta-thalassemia patients, which will be carried out at Children's Hospital & Research Center Oakland, and City of Hope.

### **About Hemoglobinopathies**

Mutations in the genes encoding beta-globin, a subunit of the oxygen-carrying protein of red blood cells, lead to the hemoglobinopathies SCD and beta-thalassemia. The mutation in beta-globin that gives rise to SCD causes the red blood cells to form an abnormal sickle or crescent shape making them adherent, fragile and less able to deliver oxygen to tissues, and they can become lodged in small blood vessels and interrupt healthy blood flow. These problems further decrease the amount of oxygen flowing to body tissues. Almost all patients with SCD have painful episodes (called crises), which can last from hours to days, and have progressive organ damage, resulting in shortened lifespan. Current standard of care is to manage and control symptoms, and to limit the number of crises. Current treatments, including blood transfusions, iron-chelation therapy and administration of hydroxyurea, pain medications and antibiotics, do not address the underlying cause of disease, and life expectancy remains substantially reduced in patients with SCD. The CDC estimates that there are currently 90,000 to 100,000 Americans living with SCD which occurs in approximately 1 out of

every 500 African-American births and 1 out of every 36,000 Hispanic-American births.

There are several forms of beta-thalassemia caused by mutations in the beta-globin gene; broadly the disorder results in excessive destruction of red blood cells leading to life-threatening anemia, enlarged spleen, liver and heart, and bone abnormalities. Beta-thalassemia major is a severe form of thalassemia that requires regular, often monthly, blood transfusions and subsequent iron-chelation therapy to treat iron overload. The CDC estimates that 2,000 people have beta-thalassemia in the United States, and an unknown number carry the genetic trait and can pass it on to their children. Thalassemia is most common among people of Mediterranean descent and is also found among people from the Arabian Peninsula, Iran, Africa, Southeast Asia and Southern China.

#### **About Biogen Idec**

Through cutting-edge science and medicine, Biogen Idec discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hemophilia and autoimmune disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company. Patients worldwide benefit from its leading multiple sclerosis therapies, and the company generates more than \$5 billion in annual revenues. For product labeling, press releases and additional information about the company, please visit [www.biogenidec.com](http://www.biogenidec.com).

#### **About Sangamo**

Sangamo BioSciences, Inc. is focused on research and development of novel DNA-binding proteins for therapeutic gene regulation and genome editing. The company has ongoing Phase 2 and Phase 1/2 clinical trials to evaluate the safety and efficacy of a novel ZFP Therapeutic® for the treatment of HIV/AIDS. As part of its acquisition of Ceregene Inc., Sangamo acquired a fully-enrolled and funded, double-blind, placebo-controlled Phase 2 trial to evaluate NGF-AAV (CERE-110) in Alzheimer's disease. Sangamo's other therapeutic programs are focused on monogenic diseases, including hemophilia, Huntington's disease and hemoglobinopathies such as sickle cell disease and beta-thalassemia. Sangamo's core competencies enable the engineering of a class of DNA-binding proteins known as zinc finger DNA-binding proteins (ZFPs). Engineering of ZFPs that recognize a specific DNA sequence enables the creation of sequence-specific ZFP Nucleases (ZFNs) for gene modification and ZFP transcription factors (ZFP TFs) that can control gene expression and, consequently, cell function. Sangamo has entered into a strategic collaboration with Shire AG to develop therapeutics for hemophilia, Huntington's disease and other monogenic diseases and has established strategic partnerships with companies in non-therapeutic applications of its technology including Dow AgroSciences and Sigma-Aldrich Corporation. For more information about Sangamo, visit the company's website at [www.sangamo.com](http://www.sangamo.com). ZFP Therapeutic® is a registered trademark of Sangamo BioSciences, Inc.

#### **Biogen Idec Safe Harbor Statement**

This press release contains forward-looking statements, including statements about Biogen Idec's expectations and goals to develop treatments for people with serious, inherited hematologic conditions, including sickle cell disease and beta-thalassemia, through its collaboration with Sangamo. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "expect," "forecast," "intend," "may," "plan," "will" and other words and terms of similar meaning. You should not place undue reliance on these statements. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including uncertainty inherent in the regulatory review process and satisfaction of the other closing conditions of the transaction, risks and uncertainties associated with drug development and commercialization, Biogen Idec's dependence on third parties over which it may not always have full control, and the other risks and uncertainties that are described in the Risk Factors section of Biogen Idec's most recent annual or quarterly report filed with the Securities and Exchange Commission. These statements are based on current beliefs and expectations and speak only as of the date of this press release. Biogen Idec does not undertake any obligation to publicly update any forward-looking statements.

#### **Sangamo Safe Harbor Statement**

This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, references to the research and development of novel ZFNs, potential therapeutic applications of the ZFN technology for the treatment of hemoglobinopathies, SCD and beta-thalassemia and potential milestone, royalty and other payments under the collaboration agreement. Actual results may differ materially from these forward-looking statements due to a number of factors, including technological challenges, uncertainties and risks relating to clinical trials, compliance with regulatory and other requirements, the ability of Sangamo to develop commercially viable products and technological developments by our competitors. See the SEC filings, and in particular, the risk factors described in Sangamo's Annual Reports on Form 10-K and most recent Quarterly Reports on Form 10-Q. Sangamo does not assume any obligation to update the forward-looking information contained in this press release.

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