Sangamo BioSciences Presents New Data at American Society of Hematology Meeting Demonstrating Broad Application of ZFN Mediated Genome-Editing Approach to Therapeutics for Hemophilia and Lysosomal Storage Disorders

Data Demonstrate Therapeutic Levels of Clotting Factor for Hemophilia in Non-Human Primates and In Vivo Protein Production of Lysosomal Storage Disease Enzymes

RICHMOND, Calif., Dec. 8, 2014 /PRNewswire/ -- Sangamo BioSciences, Inc. (Nasdaq: SGMO) announced the presentation of new preclinical data from its proprietary programs for the treatment of lysosomal storage disorders (LSD) and Shire-partnered hemophilia program. These studies demonstrate the broad applicability of Sangamo's In Vivo Protein Replacement Platform (IVPRP) for the potentially curative treatment of such diseases, and were presented at the 56th Annual Meeting of the American Society of Hematology (ASH) held in San Francisco from December 5 to 9, 2014.

The data presented at ASH from Sangamo's proprietary IVPRP applications demonstrated the efficient production, secretion and tissue uptake of functional iduronate-2-sulfatase and alpha-L-iduronidase, enzymes that are deficient in the LSDs, Hunter and Hurler's disease, respectively. Data were also presented from Sangamo's partnered program with Shire that demonstrated that therapeutic levels of Factor IX, the human clotting factor that is deficient in hemophilia B, could be generated in a dose-dependent manner in non-human primates (NHPs). There were no significant alterations in circulating albumin levels. Studies in mice also demonstrated stable Factor IX production from Sangamo's IVPRP for over 1 year.

"These data provide proof of concept for this broadly applicable genome editing strategy. We demonstrate that our process can be applied to multiple gene targets and is scalable to large animals supporting the potential application of the albumin gene as a safe harbor site for expression of therapeutic proteins," said Philip Gregory, D. Phil., Sangamo's senior
vice president of research and chief scientific officer. "In addition, data from two different LSD targets demonstrate that the enzyme product is not only efficiently produced in the liver, but secreted into the bloodstream and in a form that can be taken up by other cell types, which is important for lysosomal enzymes that function inside cells."

"These data represent an important milestone in the progress of Sangamo's monogenic disease programs towards the clinic," stated Edward Lanphier, Sangamo's president and chief executive officer. "The IVPRP is a robust and disruptive application of Sangamo's ZFN-mediated genome editing technology and can potentially be applied to any disease-relevant gene where enabling the liver to provide a stable source of corrective replacement protein will be therapeutic. The animal data presented at ASH represent a significant de-risking step and provide important proof of concept and validation for this entire strategy."

The data were presented on December 6 and 7 in an invited oral presentation entitled, "Taking it to the Clinic: Genome Editing for Blood Disorders" given by Sangamo senior scientist, Fyodor Urnov, Ph.D., as part of the session "Fixing the Broken Helix: Genome Editing for Disease Correction." During his presentation, Dr. Urnov provided an overview of the steps required to take Sangamo's genome-editing technology applications in HIV/AIDS, beta-thalassemia, hemophilia and LSDs to the clinic. Sangamo has an ongoing Phase 2 clinical trial of its ZFN-modified T-cell therapy, SB-728-T in HIV/AIDS. The Company is developing a ZFP therapeutic for beta-thalassemia with its collaborator Biogen Idec and expects to file an Investigational New Drug (IND) application for this therapeutic in 2014. In addition, Sangamo's goal is to file IND applications in 2015 for ZFP Therapeutics to address two LSDs and hemophilia.

About Sangamo's IVPRP
The IVPRP uses Sangamo's zinc finger DNA-binding protein (ZFP) genome-editing technology to enable the permanent production of therapeutic proteins from a specific genomic site in the liver with a single systemic treatment. The aim is to eliminate the requirement for repeated infusions of protein or enzyme replacement therapy (ERT) throughout the patient's life which is the current standard of care for hemophilia and LSDs. The gene encoding albumin, the most abundant protein in blood serum, was chosen as a safe harbor site because it is expressed exclusively in the liver. The albumin promoter is highly active, continuously producing large amounts of albumin protein (approximately 15g/day) which is in excess of the body's requirements. With such a large capacity for protein production, targeting and co-opting a very small percentage of the albumin gene's production capacity is sufficient to safely produce the needed replacement protein at therapeutically relevant levels with no significant effect on albumin production.

Other Sangamo Presentations at the ASH Meeting
On Monday, December 8, 2014, Sangamo scientists and collaborators will present data from research collaborations as follows:

- Abstract #308: "NY-ESO-1 Single Edited T Cells to Treat Multiple Myeloma without Inducing GvHD" Sara Mastaglio, Pietro Genovese, Zulma Magnani, Elisa Landoni, Barbara Camisa, Giulia Schirolí, Elena Provasi, Angelo Lombardo, Andreas Reik, Nicoletta Cieri, Maurilio Ponzoni, Fabio Ciceri, Claudio Bordignon, Michael C.
Holmes, Philip D. Gregory, Luigi Naldini and Chiara Bonini

- **Abstract #4796: "HPRT As a Selectable Safe Harbor for Transgenesis"**
  Anthony Conway, Josee Laganiere, David E. Paschon, Katrin Hacke, Noriyuki Kasahara, Philip D. Gregory, Michael C. Holmes and Gregory J. Cost

- **Abstract #4802: "Gene Editing of CCR5 in Hematopoietic Stem Cells in a Nonhuman Primate Model of HIV/AIDS"**
  Christopher W. Peterson, Jianbin Wang, Patricia Polacino, Michael C. Holmes, Shiu-Lok Hu, Philip D. Gregory and Hans-Peter Kiem

---

**About Sangamo**

Sangamo BioSciences, Inc. is focused on Engineering Genetic Cures™ for monogenic and infectious diseases by deploying its novel DNA-binding protein technology platform in therapeutic gene regulation and genome-editing. The Company has ongoing Phase 2 clinical trials to evaluate the safety and efficacy of a novel ZFP Therapeutic® for the treatment of HIV/AIDS (SB-728-T) and NGF-AAV for Alzheimer's disease (CERE-110). Sangamo's other therapeutic programs are focused on monogenic and rare diseases. The Company has formed a strategic collaboration with Shire International GmbH to develop therapeutics for hemophilia, Huntington's disease and other monogenic diseases, and with Biogen Idec for hemoglobinopathies, sickle cell disease and beta-thalassemia. It has also 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.**

This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, the potential of ZFNs to treat a broad range of human monogenic diseases, including beta-thalassemia, hemophilia and LSDs, research and development of novel ZFP TFs and ZFNs, therapeutic applications of Sangamo's ZFP technology platform, including IVPRP, in indications such as hemophilia and LSDs, the anticipated timing and the number of IND filings and initiation and completion of clinical trials. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs, technological challenges, Sangamo's ability to develop commercially viable products and technological developments by our competitors. For a more detailed discussion of these and other risks, please see Sangamo's SEC filings, including the risk factors described in its Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo BioSciences, Inc. assumes no obligation to update the forward-looking information contained in this press release.

Logo - [https://photos.prnewswire.com/prnh/20130102/SF35903LOGO](https://photos.prnewswire.com/prnh/20130102/SF35903LOGO)


SOURCE Sangamo BioSciences, Inc.