Sangamo BioSciences To Present Data From ZFP Therapeutic Programs At Annual Meeting Of The American Society Of Hematology

Five Presentations Include Non-Human Primate Data from Sangamo's In Vivo Protein Replacement Platform™ Approach for Hemophilia B, and Data from ZFN-Mediated Genome Editing Approach for Hemoglobinopathies

RICHMOND, Calif., Nov. 5, 2015 /PRNewswire/ -- Sangamo BioSciences, Inc. (Nasdaq: SGMO), a leader in therapeutic genome editing, announced today that non-human primate data from its proprietary In Vivo Protein Replacement Platform (IVPRP™) program for hemophilia B, and data from its ZFP Therapeutic® hemoglobinopathy programs in collaboration with Biogen, will be presented at the 57th Annual Meeting of the American Society of Hematology (ASH). The 2015 ASH meeting will be held in Orlando, FL from December 5-8, 2015.

"Our presentations at this year's ASH meeting highlight the breadth of our highly specific genome editing platform in both in vivo and ex vivo therapeutic applications," said Edward Lanphier, Sangamo's president and chief executive officer. "Data from non-human primates generated in our Factor IX program for hemophilia B demonstrate the potential of our IVPRP strategy to produce clinically beneficial levels of therapeutic protein from a single treatment. We, and our collaborators at Biogen, will also present data from our hemoglobinopathy programs in beta-thalassemia and sickle cell disease, which use an efficient ZFN-mediated knockout approach in hematopoietic stem cells to elevate functional globin to provide a potentially life-long therapeutic effect."

Sangamo's hemophilia B program is the first therapeutic application of its IVPRP strategy, an in vivo targeted integration strategy that can be leveraged across multiple monogenic diseases that are currently treated using protein or enzyme replacement therapy. Sangamo remains on track to file Investigational New Drug (IND) applications for hemophilia B (Factor IX) and Hurler syndrome (MPS I) by the end of 2015, and several
more IND applications, including hemophilia A, Hunter syndrome (MPS II), Gaucher disease and other lysosomal storage disorders in 2016.

Sangamo is collaborating with Biogen to develop a ZFP Therapeutic approach to beta-thalassemia and sickle cell disease (SCD) that replaces deficient expression of the mutant, disease-causing form of beta-globin with expression of functional fetal globin. The companies expect to file IND applications for beta-thalassemia in the first half of 2016 and for SCD in the second half of 2016.

The following presentations are scheduled at the ASH Meeting sessions:

**IVPRP**

- **ZFN-Mediated Gene Targeting at the Albumin Locus in Liver Results in Therapeutic Levels of Human FIX in Mice and Non-Human Primates** – Abstract #200
  
  **Session:** 801. Gene Therapy and Transfer: Gene Therapy for Hemoglobinopathies and Inherited Bleeding Disorders
  
  Oral Presentation – Sunday, December 6, 2015: 7:45 AM
  
  Presenter – Michael C. Holmes, Ph.D., Sangamo BioSciences

**Hemoglobinopathies**

- **Genome Editing of the Bcl11A Erythroid Specific Enhancer in Bone Marrow Derived Hematopoietic Stem and Progenitor Cells for the Treatment of Sickle Cell Disease** – Abstract #203
  
  **Session:** 801. Gene Therapy and Transfer: Gene Therapy for Hemoglobinopathies and Inherited Bleeding Disorders
  
  Oral Presentation – Sunday, December 6, 2015: 8:30 AM
  
  Presenter – Siyuan Tan, Ph.D., Biogen

- **Clinical-Scale Genome Editing of the Human BCL11A Erythroid Enhancer for Treatment of the Hemoglobinopathies** – Abstract #204
  
  **Session:** 801. Gene Therapy and Transfer: Gene Therapy for Hemoglobinopathies and Inherited Bleeding Disorders
  
  Oral Presentation – Sunday, December 6, 2015: 8:45 AM
  
  Presenter – Fyodor, D. Urnov, Ph.D., Sangamo BioSciences

- **Clonal Analysis of Human Bone Marrow CD34+ Cells Edited by BCL11A-Targeting ZFNs Reveals Clinically Relevant Levels of Gamma Globin Expression in Edited Erythroid Cells** – Abstract #3234
  
  **Session:** 801. Gene Therapy and Transfer: Poster II
  
  Poster Session – Sunday, December 6, 2015: 6:00 – 8:00 PM
  
  Presenter – Kai-Hsin Chang, Ph.D., Biogen

**Other Applications**

- **In Vivo Genome Editing in Neonatal Mouse Liver Preferentially Utilizes Homology Directed Repair** – Abstract #4422
  
  **Session:** 801. Gene Therapy and Transfer: Poster III
  
  Poster Session – Monday, December 7, 2015: 6:00 – 8:00 PM
  
  Presenter – Xavier M. Anguela, Ph.D., Children's Hospital of Philadelphia
All abstracts for the ASH meeting are available online at 2015 ASH Annual Meeting Abstracts.

**About Sangamo's IVPRP**
The IVPRP approach makes use of the albumin gene locus, a highly expressing and liver-specific genomic "safe-harbor site", that can be edited with zinc finger nucleases (ZFNs) to accept and express any therapeutic gene. The platform enables the patient's liver to permanently produce therapeutic levels of a corrective protein product such as factor VIII or IX to treat hemophilia, or replacement enzymes to treat lysosomal storage disorders. With such a large capacity for protein production (approximately 15g/day of albumin), which is in excess of the body's requirements, targeting and co-opting only a very small percentage of the albumin gene's capacity is sufficient to produce the needed replacement protein at therapeutically relevant levels with no significant effect on albumin production.

**About Sangamo's ZFP Therapeutic Approach to Hemoglobinopathies**
Sangamo's proprietary ZFN genome editing technology enables 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 causes 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 and progenitor cells (HSPCs), Sangamo's genome editing technology can be used to precisely disrupt a key DNA sequence that acts as a powerful tissue and developmental stage "Enhancer" of BCL11A expression. BCL11A is a key transcriptional regulator of the switch from fetal to adult globin production. Knockout of the Enhancer results in the disruption of that switch leading to elevation of fetal globin and reduction in the expression of adult globin.

A bone marrow transplant (BMT) of HSPCs from a "matched" related donor (allogeneic BMT) is curative for both diseases. However, this therapy is limited by 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 HSPCs that are isolated from and subsequently returned to the same patient, an autologous HSPC 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, life-long treatment for SCD and beta-thalassemia.

**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 genome editing and gene regulation. The Company has a Phase 2 clinical program to evaluate the safety and efficacy of novel ZFP Therapeutics® for the treatment of HIV/AIDS (SB-728). Sangamo's other therapeutic programs are focused on monogenic and rare diseases. The Company has formed a strategic collaboration with Biogen Inc. for hemoglobinopathies, such as sickle cell disease and beta-thalassemia, and with Shire International GmbH to develop therapeutics for Huntington's disease. 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.
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, sickle cell disease, hemophilia A and B, Hurler syndrome, Hunter syndrome, Gaucher disease and other 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, the collaboration with Biogen 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.

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