Sangamo BioSciences Announces New Gene Therapy Clinical Development Program for Treatment Of Hemophilia A

Scientists Present Preclinical Data Demonstrating High Levels of Factor VIII Protein Expression from Proprietary Therapeutic AAV cDNA Construct at World Federation of Hemophilia 2016 World Congress


RICHMOND, Calif., July 26, 2016 /PRNewswire/ -- Sangamo BioSciences, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, today announced the presentation of preclinical data that supports the clinical development of its new proprietary gene therapy for the treatment of hemophilia A. This new therapeutic comprises an adeno-associated virus (AAV) cDNA human Factor 8 (hF8) construct driven by Sangamo's proprietary synthetic liver specific promoter, which in preclinical studies is at least three times more potent than existing AAV-based cDNA constructs currently under evaluation for the treatment of hemophilia A.

"We have compelling preclinical data from this AAV cDNA therapeutic which suggest its potential to be 'best in class' in this highly competitive field," said Geoff Nichol, M.B., Ch.B., Sangamo's executive vice president of research and development. "As a leading science and data-driven company, Sangamo has the expertise to efficiently move new technological advances into our therapeutic portfolio. We intend to move this cDNA gene therapy approach for hemophilia A into the clinic as soon as possible while continuing to optimize our in vivo zinc finger nuclease (ZFN) genome editing approach, which may be more suitable for certain groups of hemophilia A patients."

The data, presented by Sangamo scientists at the World Federation of Hemophilia (WFH) 2016 World Congress, being held in Orlando, Florida, from July 24 – 28, 2016, demonstrated production of supraphysiologic levels of human Factor VIII (hFVIII) in a mouse model of the disease and in non-human primates (NHPs). Mean levels of hFVIII several fold in excess of normal, obtained using research grade AAV in NHPs, were
confirmed by dosing with AAV manufactured using Sangamo's clinical process. In these animals, mean hFVIII levels ranged from 5 - 230% of normal and were obtained using AAV doses in the $6 \times 10^{11} - 6 \times 10^{12}$ vgs/kg range - the most potent dose response in NHPs thus far disclosed for an hF8 cDNA. Based on other studies in the field, vector dose and corresponding hFVIII expression levels observed in NHPs are highly predictive of those observed in patients. The high potency of this novel therapeutic may enable clinically relevant levels of hFVIII to be obtained using lower vector doses, which potentially provides a better therapeutic risk/benefit profile for patients. Sangamo is conducting additional studies to determine the minimal effective dose necessary to provide therapeutic benefit.

"As we continue our evolution from a platform company to a clinical-stage therapeutic product company, we will capitalize on our extensive knowledge and expertise in genome editing and gene therapy to develop the best therapeutic options for patients," said Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo's president and chief executive officer. "The development of AAV cDNA gene therapy approaches for hemophilia A has been challenging due to a number of factors, including the large size of the native hF8 gene, low levels hFVIII gene expression from conventional promoters and low yields of vector in large scale manufacturing processes. Our scientists have designed a superior therapeutic AAV cDNA construct that has the potential to overcome all of these issues."

"The synergy of our technology capabilities in both gene delivery and genome editing enables us to diversify our therapeutic portfolio to include conventional gene therapy products as well as our ZFN-based genome editing products," continued Dr. Macrae. "Our strategy is to develop a portfolio of highly differentiated therapeutics. We will continue to leverage our core competencies in both gene therapy and therapeutic genome editing across relevant indications within our pipeline, expanding our capabilities in manufacturing, clinical development and new product commercialization. In hemophilia A specifically, we plan to develop a portfolio designed to address the specific needs and characteristics of different patient populations, including the prevalent and incident populations."

The Company will focus on accelerating clinical development of its AAV cDNA approach for the treatment of hemophilia A with the goal of filing an investigational new drug (IND) application with the U.S. Food and Drug Administration (FDA) in 2016.

Sangamo expects to provide updated guidance for its in vivo genome editing approach for hemophilia A and other programs during its second quarter 2016 conference call.

About Hemophilia A
Hemophilia, a rare bleeding disorder in which the blood does not clot normally, is caused by mutations in genes that encode factors which help the blood clot and stop bleeding when blood vessels are injured. Hemophilia A is caused by a defect in the gene encoding Factor VIII protein, which is involved in clotting, and individuals with this mutation experience bleeding episodes after injuries and spontaneous bleeding episodes that often lead to destructive joint disease. According to the U.S. Centers for Disease Control and Prevention, hemophilia A occurs in about one in 5,000 live births. There are about 16,000 people living with hemophilia A in the U.S., with more than half of patients having the
severe form of the disease. The standard treatment for individuals with hemophilia is replacement of the defective clotting factor with regular, often frequent infusions of recombinant clotting factors or plasma concentrates. These therapies are expensive and sometimes stimulate the body to produce antibodies against the factors that inhibit the benefits of treatment. The most severe forms of hemophilia A require the need for ongoing, preventive infusions.

About World Federation of Hemophilia
The World Federation of Hemophilia (WFH) is an international non-profit organization established in 1963. It is a global network of patient organizations in 127 countries, including the National Hemophilia Foundation in the U.S., the Canadian Hemophilia Society, and the U.K. Haemophilia Society, and has official recognition from the World Health Organization. For over 50 years, the WFH has provided global leadership to improve and sustain care for people with inherited bleeding disorders, including hemophilia, von Willebrand disease, rare factor deficiencies and inherited platelet disorders.

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's proprietary In Vivo Protein Replacement Platform™ (IVPRP) approach is focused on monogenic diseases, including hemophilia and lysosomal storage disorders. Based on its proprietary IVPRP approach, Sangamo is initiating Phase 1/2 clinical trials for hemophilia B, the first in vivo genome editing application cleared by the FDA, MPS I and MPS II. In addition, Sangamo has a Phase 2 clinical program to evaluate the safety and efficacy of novel ZFP Therapeutics® for the treatment of HIV/AIDS (SB-728). The Company is also developing an adeno-associated viral (AAV) gene therapy product for the treatment of hemophilia A, based on its proprietary vector construct. Sangamo has also 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 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.

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, references relating to research and development of novel ZFNs, AAV gene therapy vectors and therapeutic applications of Sangamo's technology platform; the potential of Sangamo's genome editing and gene therapy technologies to treat hemophilia A, and the safety of the approach of using AAV cDNA gene therapy products in vivo; the expected filing of IND applications and initiation of clinical trials for the hemophilia A program; and the applicability of Sangamo's ZFP technology in monogenic diseases. 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 safety, tolerability and efficacy of ZFNs, AAV gene therapy vectors, 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 public filings with the Securities and Exchange Commission, including the risk factors described in its Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

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