Sangamo BioSciences Announces In-Licensing Of mRNA Delivery Technology And Expansion Of Therapeutic Pipeline Opportunities For Sangamo's In Vivo ZFN Genome-Editing Platform

January 8, 2015

mRNA Delivery a Natural Fit for ZFN-mediated Genome Editing Which Requires Only Transient Expression to Achieve a Permanent Therapeutic Effect

RICHMOND, Calif., Jan. 8, 2015 /PRNewswire/ -- Sangamo BioSciences, Inc. (Nasdaq: SGMO) announced that the company has in-licensed nanoparticle technology to enable systemic messenger RNA (mRNA) delivery of Sangamo's proprietary zinc finger nucleases (ZFNs). mRNA delivery of ZFNs is being used in the development of the next generation of Sangamo's ZFP Therapeutics®.

"We continue to identify strategies to leverage and maximize the value of our ZFN platform by enhancing and broadening the application of Sangamo's ZFP technology into new therapeutic opportunities," stated Edward Lanphier, Sangamo's president and CEO. "Nucleic acid delivery technologies have significantly matured over the past several years and mRNA delivery is coming of age. While we already employ mRNA delivery in our *ex vivo* genome editing programs, novel mRNA formulations potentially enable *in vivo* ZFN applications such as the knock-out of well-established repression targets in the liver."

"Specifically, this strategy opens up an entirely new set of therapeutic genome-editing targets," added Mr. Lanphier. "mRNA delivery could enable progressive and permanent knock out of a gene at the DNA level versus repressing its expression using continued and chronic administration of antisense or RNAi therapeutics."

Because RNA is rapidly turned over in cells and tissues, conventional RNA-based drugs such as antisense and RNAi must be repeatedly administered to maintain a therapeutic effect. In contrast, ZFNs need to be present only transiently to enable permanent changes in the genome of cells, and so are ideally suited for mRNA delivery.

This delivery mode has several advantages. The potential to dose patients multiple times provides the opportunity to "dose to effect" or to administer the ZFNs in several treatments until a sufficient level of permanent modification is obtained to achieve a life-long therapeutic outcome.

This method potentially expands the application of ZFN *in vivo* genome-editing to numerous therapeutic "knock-out" targets such as *PCSK9*, which encodes a protein involved in cholesterol homeostasis. Knocking out the *PCSK9* gene is expected to lower levels of low-density lipoprotein cholesterol (LDL-C), or "bad" cholesterol, which is considered a risk factor for cardiovascular disease.

In addition, the mRNA delivery method could be used in the development of second generation ZFP Therapeutics to broaden the patient group that can be treated using Sangamo's In Vivo Protein Replacement Platform (IVPRP) which is being developed as a generally applicable strategy to provide a permanent genetic cure for certain monogenic diseases.

Background on Sangamo's IVPRP Approach

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

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, such as 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.

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 mRNA technologies to expand the therapeutic application of ZFNs, research and development of novel ZFP TFs and ZFNs, their delivery, and therapeutic applications of Sangamo's ZFP technology platform for the treatment of monogenic and infectious diseases. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to technological challenges, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs and ZFP TFs, 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.

Logo - https://photos.prnewswire.com/prnh/20130102/SF35903LOGO

SOURCE Sangamo BioSciences, Inc.

Released January 8, 2015