Sangamo Therapeutics Presents Latest Advances In Zinc Finger Nuclease Platform Technology At Keystone Symposium On Precision Genome Engineering

- New architectures enable targeting of any chosen location in the genome with very high precision

- New framework variants and design strategies for highly specific cleavage

- On-target modification levels of 80% demonstrated in T cells and hematopoietic stem and progenitor cells with no significant off-target cleavage

RICHMOND, Calif., Jan. 10, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), the leader in therapeutic genome editing, today announced the presentation of key improvements to its technology platform for engineering highly specific zinc finger nucleases (ZFNs) for therapeutic genome editing applications. The presentation was delivered January 9, 2017 by Edward Rebar, Ph.D., Sangamo's vice president, technology, an invited speaker at the Keystone Symposium on Precision Genome Engineering, which is being held this week in Breckenridge, CO.

The data demonstrate genome editing of therapeutic gene targets in clinically relevant cell types, including high levels of targeted modification (80%) of the BCL11A enhancer in hematopoietic stem and progenitor cells at clinical scale with no significant off-target activity. Off-target activity was assessed using state of the art assays involving unbiased oligonucleotide capture methods followed by deep sequencing.

"Based on a human DNA-binding motif, we believe ZFNs are the most advanced, flexible and precise genome editing technology available, and, as our scientists have
demonstrated, they can be further engineered to refine their specificity and potency for therapeutic use," said Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo's CEO. "The new architectures and strategies that we describe represent a substantial improvement in our capabilities while retaining full compatibility with our existing genome editing platform. The modifications enable us to target any investigator chosen site in the genome with enhanced precision and a reduction in off-target cleavage to levels that are at or below the limit of detection even under conditions of very high on-target activity. Importantly, these results are achievable at clinical scale, using clinical delivery conditions, and in clinically relevant cell types."

ZFNs can be engineered to introduce a precise double strand break at any chosen location in the genome to correct, delete or add genes. The advances described in Dr. Rebar's talk include new linkers for connecting individual fingers to each other or to the DNA cleavage domain, which enables greater configurational diversity and increases the number of sites in the genome that can be targeted. Modifications of key framework residues in the zinc fingers are also presented that can selectively suppress off-target cleavage activity by >100 fold.

"These engineering advances are exciting because they enable development of extremely specific nucleases for therapeutic applications that are not limited by the targeting constraints inherent to other cleavage platforms," said Dr. Rebar. "These new architectures will also accelerate development of highly active and specific ZFNs, resulting in much faster identification of therapeutic leads."

In addition to the Keystone Symposium, Sangamo is also participating this week in the JP Morgan Healthcare Conference being held in San Francisco. Sangamo CEO Sandy Macrae will present an overview of the Company's strategy, clinical development programs, and research on January 11th, 2017 at 4:30 pm Pacific Time. A live webcast of Dr. Macrae's presentation will be accessible through a link on the Investors + Media section of the company's website, www.sangamo.com.

About Sangamo
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company's proprietary zinc finger nuclease (ZFN) in vivo genome editing approach is being evaluated in Phase 1/2 clinical trials to treat hemophilia B and lysosomal storage disorders MPS I and MPS II. Sangamo is also conducting a Phase 1/2 clinical trial to evaluate its AAV cDNA human Factor 8 gene therapy approach, SB-525, to treat hemophilia A. Sangamo has a strategic collaboration with Biogen, Inc. for hemoglobinopathies, including sickle cell disease and beta-thalassemia, and with Shire plc to develop therapeutics for Huntington's disease. In addition, Sangamo has Phase 1/2 and Phase 2 clinical programs in HIV/AIDS (SB-728). 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.

Forward Looking Statements
This press release may contain forward-looking statements based on Sangamo’s current expectations. These forward-looking statements include, without limitation, the advantages and capabilities of improved ZFN engineering in therapeutic applications, the potential of modified ZFN technology to enhance clinical development and identification of therapeutic leads, and the ability of the new ZFN architectures to enable specific modification of DNA with high efficiency without significant off-target event. 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 Therapeutics, Inc. assumes no obligation to update the forward-looking information contained in this press release.


SOURCE Sangamo Therapeutics, Inc.