

Sangamo Therapeutics Announces Presentations at 2017 Annual meeting of the American Society of Gene & Cell Therapy

April 24, 2017

RICHMOND, Calif., April 24, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, announced that data from the Company's therapeutic and research programs will be presented at the 20th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) to be held in Washington, D.C. from May 10-13, 2017.



Sangamo scientists or collaborators will deliver ten oral and nine poster presentations during the conference. These presentations will detail data from therapeutic and research programs for lysosomal storage disorders and other monogenic diseases, cancer immunotherapy, and central nervous system disorders, as well as advancements in genome editing technology and novel delivery modalities. Sangamo scientists and their collaborators have also been invited to participate in scientific symposia and educational sessions focused on clinical and research applications of genome editing.

"Sangamo once again has a very strong presence at ASGCT, with 19 oral and poster presentations," said Dr. Sandy Macrae, Sangamo's chief executive officer. "These data highlight the breadth of our clinical and early stage pipeline across genome editing, gene therapy, gene regulation and cell therapy. With our focus now on the translation of our groundbreaking science into new genomic therapies that transform patients' lives, our research and technology programs will continue to provide new assets for therapeutic development."

The following presentations are scheduled at the ASGCT Meeting sessions:

Invited Presentations at Scientific Symposia

- *C-Suite Executive Panel: Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo Therapeutics*
Session: Commercialization Workshop
Panel Discussion – Tuesday, May 9; 3:15PM
- *Preclinical Studies Evaluating Zinc Finger Nuclease-Driven Genome Editing – Michael C. Holmes, Ph.D., Sangamo Therapeutics*
Session: Clinical Trials Training Course
Invited Talk – Tuesday, May 9; 9:50AM
- *Educational Session Co-Chair: Thomas Wechsler, Ph.D., Sangamo Therapeutics*
Session: 100. Getting Started in Genome Editing
Panel Discussion – Wednesday, May 10; 8:00AM
- *Scientific Symposium Co-Chair: Michael C. Holmes, Ph.D., Sangamo Therapeutics*
Session: 204. Therapeutic Editing of the Human Genome and Epigenome
Panel Discussion – Thursday, May 11; 8:00AM
- *Scientific Symposium Co-Chair: Kathleen Meyer, M.P.H., Ph.D., D.A.B.T., Sangamo Therapeutics*
Session: 300. Clinical Advancement of Gene Editing – Moving New Science to the Clinic
Panel Discussion – Friday, May 12; 8:00AM

Lysosomal Storage Disorders

- *Liver-Based Expression of the Human alpha-Galactosidase A Gene in a Murine Fabry Model Results in Continuous High, Therapeutic Levels of Enzyme Activity and Effective Substrate Reduction – Abstract #27*
Session: 113. Genome Editing and Integration Analysis in Metabolic and Endocrine Disorders
Oral Presentation – Wednesday, May 10; 10:45AM
- *ZFN-Mediated In Vivo Genome Editing Results in Phenotypic Correction in MPS I and MPS II Mouse Models – Abstract #30*
Session: 113. Genome Editing and Integration Analysis in Metabolic and Endocrine Disorders
Oral Presentation – Wednesday, May 10; 11:30AM

Central Nervous System Disorders

- *Sustained Tau Reduction via Zinc Finger Protein Transcription Factors as a Potential Next-Generation Therapy for Alzheimer's Disease and Other Tauopathies – Abstract #24*
Session: 112. Genome Editing: Transcriptional Regulation and Specificity

Oral Presentation – Wednesday, May 10; 12:00PM

Monogenic and Infectious Diseases

- *In Vivo ZFN-Mediated Editing of the Mutant SERPINA1 Gene Results in Spontaneous Liver Repopulation by the Gene-Edited Hepatocytes and Greatly Decreased Fibrosis in the PiZ Mouse Model of alpha-1 Antitrypsin Deficiency Liver Disease – Abstract #511*

Session: 341. In Vivo Gene Editing

Oral Presentation – Friday, May 12; 4:15PM

- *Targeted Genome Editing of Recombination Activating Gene 1 to Potentially Treat Severe Combined Immunodeficiency – Abstract #654*

Session: Gene Targeting and Gene Correction III

Poster Presentation – Friday, May 12; 5:45PM

- *Evolution of HIV-1 Resistance to the Fusion Inhibitor CD34-CXCR4 and Potential Fitness Costs in Consideration of a Phase 1 Clinical Trial – Abstract #657*

Session: Hematologic & Immunologic Diseases III

Poster Presentation – Friday, May 12; 5:45PM

Technology and Delivery Developments

- *New Zinc Finger Nuclease Architectures for Highly Efficient Genome Engineering in Primary Cells at Large Scale with No Detectable Off-Target Effects – Abstract #23*

Session: 112. Genome Editing: Transcriptional Regulation and Specificity

Oral Presentation – Wednesday, May 10; 11:45AM

- *In Vivo Genome Editing via Non-Viral Delivery of Zinc Finger Nucleases Results in Supraphysiological Levels of Therapeutic Proteins in Adult Mice – Abstract #509*

Session: 341. In Vivo Gene Editing

Oral Presentation – Friday, May 12; 3:45PM

- *Non-Viral Delivery of Zinc Finger Nucleases Enable Greater Than 90% Protein Knockdown of Multiple Therapeutic Gene Targets In Vivo – Abstract #510*

Session: 341. In Vivo Gene Editing

Oral Presentation – Friday, May 12; 4:00PM

- *Ex Vivo Protein Replacement Using Homology Driven Genome Editing in Human B Cells by Combining Zinc Finger Nuclease mRNA and AAV6 Donor Delivery – Abstract #750*

Session: 412. Ex Vivo Gene Editing

Oral Presentation – Saturday, May 13; 11:30AM

- *A New, Reversed Zinc-Finger Nuclease Structure for High-Precision Therapeutic Genome Engineering – Abstract #170*

Session: Gene Targeting and Gene Correction I

Poster Presentation – Wednesday, May 10; 5:30PM

- *Improved In Vitro Assay to Assess Human Serum Neutralization of AAV Vectors Yields Cell Line-Dependent Results – Abstract #396*

Session: Immunological Aspects of Gene Therapy and Vaccines II

Poster Presentation – Thursday, May 11; 5:15PM

- *Development of a Qualifiable MiSeq Assay for Precise and Accurate Quantitation of Small Insertions and Deletions (Indels) in the Human Genome Induced by Sequence-Specific Zinc Finger Nucleases – Abstract #644*

Session: Gene Targeting and Gene Correction III

Poster Presentation – Friday, May 12; 5:45PM

Applications of Gene Editing in Stem Cells

- *In Vivo Selection of Engineered Human CD34⁺ HSPCs Using Targeted Gene Integration – Abstract #512*

Session: 341. In Vivo Gene Editing

Oral Presentation – Friday, May 12; 4:30PM

- *Correction of SCID-X1 by Targeted Genome Editing of Hematopoietic Stem/Progenitor Cells (HSPC) in a Humanized Mouse Model – Abstract #747*

Session: 412. Ex Vivo Gene Editing

Oral Presentation – Saturday, May 13; 10:45AM

- *A Novel Gene Therapy Approach of Fanconi Anemia Hematopoietic Stem Cells Based on NHEJ-Mediated Gene Editing – Abstract #165*

Session: Gene Targeting and Gene Correction I

Poster Presentation: Wednesday, May 10; 5:30PM

- *Towards Clinical Translation of Hematopoietic Stem Cell Gene Editing for the Correction of SCID-X1 Mutations – Abstract #163*
Session: Gene Targeting and Gene Correction I
Poster Presentation: Wednesday, May 10; 5:30PM
- *HSPC Expansion Drugs Enhance Gene Editing Efficiency in Long Term Hematopoietic Stem Cells – Abstract #378*
Session: Gene Targeting and Gene Correction II
Poster presentation: Thursday, May 11; 5:15PM
- *Molecular Evidence of Ex Vivo Genome Editing in a Mouse Model of Immunodeficiency – Abstract #656*
Session: Hematologic & Immunologic Diseases III
Poster Presentation – Friday, May 12; 5:45PM

All abstracts for the ASGCT meeting are available online at [2017 ASGCT Annual Meeting Abstracts](#).

About Sangamo Therapeutics

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 is advancing Phase 1/2 clinical programs in hemophilia A and hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo has a strategic collaboration with Bioverativ Inc. for hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at www.sangamo.com.

Forward Looking Statements

This press release contains forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, references relating to presentation of data from various therapeutic and research programs and the potential of these programs to transform the lives of patients. These statements are not guarantees of future performance and are subject to certain risks, uncertainties and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the dependence on the success of clinical trials of lead programs, the lengthy and uncertain regulatory approval process, uncertainties related to the timing of initiation and completion of clinical trials, whether clinical trial results will validate and support the safety and efficacy of ZFP Therapeutics, and the ability to establish strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals will be obtained or that Sangamo and its partners will be able to develop commercially viable gene-based therapeutics. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q as filed with the Securities and Exchange Commission. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/sangamo-therapeutics-announces-presentations-at-2017-annual-meeting-of-the-american-society-of-gene--cell-therapy-300444609.html>

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