Sangamo Therapeutics Logo

Sangamo Therapeutics Announces Presentations At 2019 Annual Meeting Of The American Society Of Gene & Cell Therapy

April 15, 2019

BRISBANE, Calif., April 15, 2019 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), a genomic medicine company, today announced that Sangamo scientists and collaborators will present data from the Company's clinical and preclinical pipeline at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) being held April 29th to May 2nd, 2019 in Washington, D.C.

Abstracts accepted for presentation highlight data from the Company's gene therapy program in Fabry disease; *ex vivo* gene-edited cell therapy programs in hemoglobinopathies being developed in partnership with Sanofi; and preclinical programs for CNS diseases using Sangamo's gene regulation approach, which include tauopathies, C9ORF72-linked ALS in collaboration with Pfizer, and Huntington's disease in collaboration with Takeda.

"Sangamo's strong scientific presence at ASGCT demonstrates the breadth of our therapeutic pipeline and our expertise in innovative genomic medicines research and development," said Adrian Woolfson, M.D., Ph.D., Sangamo's Executive Vice President of Research and Development. "Through our pioneering work in developing zinc fingers for *ex vivo* and *in vivo* genome editing, we've gained invaluable insights into AAV manufacturing and delivery, as well as gene editing precision, efficiency, and specificity. We're now applying these insights to our integrated portfolio of programs in both gene therapy and *ex vivo* cell therapy as well as to our new zinc finger protein transcription factor gene regulation platform, which will be showcased at this year's meeting."

ASGCT Annual Meeting presentations

Gene Therapy

- *Liver-Targeted AAV Gene Therapy Vectors Produced at Clinical Scale Result in High, Continuous Therapeutic Levels of α-GalA Enzyme Activity and Effective Substrate Reduction in a Mouse Model of Fabry Disease – Abstract #794*
  Session: Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases II
  Poster Presentation – Wednesday, May 1; 5:00PM ET

***Ex Vivo* Gene-Edited Cell Therapy**

- *Zinc Finger Nuclease-Mediated Disruption of the BCL11A Erythroid Enhancer in Human Hematopoietic Stem and Progenitor Cells Results in Enriched Bialleleic Editing with Highly Replicable and Precise On-Target Small Indels and Allele-Additive Increases in Fetal Hemoglobin – Abstract #972*
  Session: Gene Editing for Red Blood Cell Disorders
  Oral Presentation – Thursday, May 2; 11:00AM ET

Zinc Finger Nuclease Engineering

- *Optimizing Nuclease Specificity via Catalytic Domain Engineering Enables Complete Gene Modification with No Detectable Off-Targets – Abstract #133*
  Session: Measuring and Mitigating Genotoxicity of Genome Editing
  Oral Presentation – Monday, April 29; 4:00PM ET

Gene Regulation

- *Evaluation of Programmable Zinc Finger Protein Transcription Factors for the Efficient Reduction of Tau in the Nonhuman Primate Brain – Abstract #62*
  Session: Gene Silencing Approaches
  Oral Presentation – Monday, April 29; 10:45AM ET
- *Repression of mHTT Expression in Huntington's Disease Mouse Models by AAV-Mediated Expression of Zinc-Finger Protein-Repressor Transgene – Abstract #63*
  Session: Gene Silencing Approaches
  Oral Presentation – Monday, April 29; 11:00AM ET
- *Selective Repression of C90RF72 Repeat Expansion-Containing Transcripts for the Treatment of ALS – Abstract #555*
  Session: Neurologic Diseases II

Poster Presentation – Tuesday, April 30; 5:00PM ET

Invited Presentations at Scientific Symposia

Sangamo's research and development team and clinical collaborators have additionally been invited to participate in a variety of special workshops and scientific symposia. Dr. Ed Rebar, Sangamo's chief technology officer, will present an overview of preclinical and clinical programs in the Gene Editing Workshop program and later in the week will discuss stem cell modification for the treatment of hemoglobinopathies. Dr. Paul Harmatz, a professor at UCSF Benioff Children's Hospital Oakland and a principle investigator for Sangamo's *in vivo* genome editing clinical programs in mucopolysaccharidosis type I (MPS I) and MPS II, will review clinical data presented earlier this year at the 2019 WORLD*Symposium*. Dr. Sagar Vaidya, a senior medical director at Sangamo, will discuss operational aspects of genomic medicine clinical trials.

- *Therapeutic Genome Editing and Gene Regulation Using Designed Zinc Finger Proteins – Ed Rebar, Ph.D., Sangamo Therapeutics*
  Session: Gene Editing Workshop
  Invited Talk – Sunday, April 28; 5:00PM ET
- *Clinical Trials of Genome-editing (ZFN) for MPS I and MPS II – Paul Harmatz, M.D., UCSF Benioff Children's Hospital Oakland*
  Session: Novel Strategies for Lung and GI Tract-directed Genetic Therapies – Organized by the Respiratory & GI Tract Gene & Cell Therapy Committee
  Invited Talk – Monday, April 29; 9:00AM ET
- *Gene Modification of Hematopoietic Stem Cells for Research and Treatment of Hemoglobinopathies – Ed Rebar, Ph.D., Sangamo Therapeutics*
  Session: Genetic Modification of Stem Cells – Organized by the Stem Cell Committee
  Invited Talk – Wednesday, May 1; 9:30 AM ET
- *IRB and Patient Safety 5 Things You Must Know to Start a Human Gene Therapy Trial – Sagar Vaidya, M.D., Ph.D., Sangamo Therapeutics*
  Session: Getting to the Finish Line: Market Success – Organized by the Clinical Trials and Regulatory Affairs Committee
  Invited Talk – Thursday, May 2; 8:30AM ET

All abstracts for the ASGCT Annual Meeting are available online at 2019 ASGCT Annual Meeting Abstracts.

About Sangamo Therapeutics
Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic medicines with the potential to transform patients' lives using gene therapy, *ex vivo* gene-edited cell therapy, *in vivo* genome editing, and gene regulation. For more information about Sangamo, visit 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 third parties for clinical trial supply and conduct of clinical trials, the lengthy and uncertain regulatory approval process, uncertainties related to the timing of initiation, enrollment and completion of clinical trials, and whether clinical trial results will validate and support the safety and efficacy of our product candidates. 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 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. These risks and uncertainties are described more fully in Sangamo's Annual Report on Form 10-K and its Current Report on Form 8-K 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.

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