



## **Sangamo Therapeutics to Present Neurology-Focused Pre-Clinical Data From Its Epigenetic Regulation, Capsid Delivery and Genome Engineering Platforms at the 27th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)**

April 22, 2024

RICHMOND, Calif.--(BUSINESS WIRE)--Apr. 22, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the American Society of Gene & Cell Therapy (ASGCT) has accepted 20 Sangamo abstracts for presentation at the 27<sup>th</sup> ASGCT Annual Meeting being held May 7-11, 2024, in-person in Baltimore, MD and in a virtual format. Presentations will focus on the progression of Sangamo's neurology-focused pre-clinical pipeline, including data supporting innovations in zinc finger epigenetic regulation, advances in AAV capsid engineering, and discovery of next-generation integrase technology.

"We are thrilled to be showcasing at ASGCT the strong depth of our neurology pipeline, including various applications for our zinc finger epigenetic regulation platform, exciting advances in our capsid delivery technology, and new, potentially transformative Modular Integrases (MInts), that together reflect our continued commitment to developing potential neurology genomic medicines for patients in need," said Amy Pooler, Head of Research at Sangamo. "Potent epigenetic regulation in diseases such as prion disease and tauopathies are complemented by our exceptional AAV delivery capabilities, a combination that is essential to the development of effective potential genomic medicine treatments to treat devastating neurological conditions."

"These data showcase the deep expertise of our scientific team in engineering DNA-protein interactions and the broad potential reach of our innovative pre-clinical approaches, including exciting new discoveries for site-directed integration of large DNA sequences via engineered MInts," said Greg Davis, Head of Technology at Sangamo. "Using compact, protein-guided integrases built from Bxb1 variants as a means of targeted integration expands our genome editing abilities and underscores our drive to develop technologies that enable potentially transformative new medicines."

Data presentations at the ASGCT Annual Meeting include three oral presentations, two of which detail Sangamo's potent delivery capabilities developed through its AAV capsid engineering platform SIFTER, and one that will discuss a new breakthrough in programming Bxb1, a large serine recombinase, for the development of MInts to potentially enable large-scale genome engineering. These data will showcase a novel AAV capsid, STAC-BBB, that exhibited robust and widespread central nervous system transduction in cynomolgus macaques after intravenous delivery, as well as another novel AAV capsid, STAC-150, engineered to accelerate the discovery of potent and highly specific epigenetic regulators. Sangamo will also showcase data highlighting the use of MInts, derived from compact Bxb1 variants, to integrate large sequences of DNA into the genome with minimal double-stranded DNA breaks and without the need for assistance from ancillary genome editing or DNA-repair modulating cargo.

Additional poster presentations at the ASGCT Annual Meeting will showcase the use of zinc finger activators and repressors for the potential treatment of neurological diseases such as prion disease, tauopathies, Charcot-Marie-Tooth disease type 1A (CMT1A), Dravet Syndrome, SOD1-mediated amyotrophic lateral sclerosis (ALS), Phelan-McDermid syndrome, Parkinson's disease, Angelman syndrome, and other CNS disorders. Sangamo will also present additional data from its AAV capsid engineering platform SIFTER and its efforts for integrase evolution and utilization.

### **ASGCT Annual Meeting Presentations and Invited Sessions**

#### **Epigenetic Regulation for the Central Nervous System**

- Restoration of Normal Gene and Protein Expression in Mouse and Human Disease Models of SCN2A Haploinsufficiency Using Zinc Finger Activators
  - Abstract No. 636
  - Poster Presentation – May 8; 5:30-7:00 pm ET
- A Zinc Finger Activator Platform to Restore Normal Gene & Protein Expression in Cellular Models of Dravet Syndrome
  - Abstract No. 642
  - Poster Presentation – May 8; 5:30-7:00 pm ET
- SNCA Gene Repression Mediated by Zinc Finger Repressors (ZFRs) as a Therapeutic Approach for Parkinson's Disease
  - Abstract No. 1120
  - Poster Presentation – May 9; 5:30-7:00 pm ET
- UBE3A Gene Activation Mediated by Zinc Finger Activators (ZFAs) as a Therapeutic Approach for Angelman Syndrome
  - Abstract No. 1121
  - Poster Presentation – May 9; 5:30-7:00 pm ET
- Whole CNS Human Tau Knockdown for the Potential Treatment of Alzheimer's Disease and Other Tauopathies
  - Abstract No. 1126
  - Poster Presentation – May 9; 5:30-7:00 pm ET
- SOD1 Gene Repression Mediated by Zinc Finger Repressors (ZFRs) as a Therapeutic Approach for SOD1-Mediated ALS
  - Abstract No. 1597
  - Poster Presentation – May 10; 5:30-7:00 pm ET
- PMP22 Gene Repression Mediated by Zinc Finger Repressors (ZFRs) as a Therapeutic Approach for CMT1A
  - Abstract No. 1600

- Poster Presentation – May 10; 5:30-7:00 pm ET
- Shank3 Gene Activation Mediated by Zinc Finger Transcriptional Activators (ZFA) as a Therapeutic Approach for Phelan-McDermid Syndrome
  - Abstract No. 1605
  - Poster Presentation – May 10; 5:30-7:00 pm ET
- Development of a Robust Zinc Finger Activation Platform for Treatment of Neurological Disorders
  - Abstract No. 1609
  - Poster Presentation – May 10; 5:30-7:00 pm ET
- Epigenetic Regulation of Human Prion Expression as a Potential One-Time Treatment for Prion Disease
  - Abstract No. 1616
  - Poster Presentation – May 10; 5:30-7:00 pm ET

#### **Viral Engineering for the Central Nervous System**

- Identification and Characterization of STAC-BBB, an Engineered AAV Capsid That Exhibits Widespread Transduction of the Central Nervous System in Cynomolgus Macaques
  - Abstract No. 117
  - Oral Presentation – May 8; 4:00-4:15 pm ET
- A Highly Potent Engineered AAV Capsid, STAC-150, Enables High-Throughput AAV Production and Arrayed Epigenetic Regulator Screening Directly in Cultured Neurons
  - Abstract No. 351
  - Oral Presentation – May 10; 5:15-5:30 pm ET
- Optimal Drug Product Presentation and Container Closure Selection for AAV-Based Genomic Medicines
  - Abstract No. 547
  - Poster Presentation – May 8; 5:30-7:00 pm ET
- Development of Blood-Brain Barrier Penetrant AAVs through Receptor-Targeted Capsid Engineering
  - Abstract No. 985
  - Poster Presentation – May 9; 5:30-7:00 pm ET
- Unraveling Impact of Manufacturing Process-Related Stresses on AAV Stability, Aggregation, and DNA Release
  - Abstract No. 1032
  - Poster Presentation – May 9; 5:30-7:00 pm ET
- Process and Formulation Development for a Novel Blood-Brain Barrier Penetrant AAV Capsid
  - Abstract No. 1052
  - Poster Presentation – May 9; 5:30-7:00 pm ET

#### **Next-Generation Genome Engineering**

- Directed Evolution of Bxb1 for the Development of Modular Integrases (MInts)
  - Abstract No. 192
  - Oral Presentation – May 9; 4:00-4:15 pm ET
- Zinc Finger Mediated Repression and Replacement of MFN2 Leads to the Rescue of Cellular Disease Phenotype in CMT2A Patient-Derived Cells
  - Abstract No. 637
  - Poster Presentation – May 8; 5:30-7:00 pm ET
- Highly Specific Zinc Finger Proteins with Synthetic Target Sites Enable Self-Regulated Expression of Dosage-Sensitive Transgenes
  - Abstract No. 722
  - Poster Presentation – May 8; 5:30-7:00 pm ET
- Site-directed integration of large DNA sequences into endogenous sites in the human genome using engineered Modular Integrases (MInts)
  - Abstract No. 1680
  - Poster Presentation – May 10; 5:30-7:00 pm ET

All abstracts for the ASGCT Annual Meeting are available on [ASGCT's website](#).

#### **About Sangamo Therapeutics**

Sangamo Therapeutics is a genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases who do not have adequate or any treatment options. Sangamo's zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and Sangamo's capsid discovery platform is expanding delivery beyond currently available intrathecal delivery capsids, including in the central nervous system. Sangamo's pipeline also includes multiple partnered programs and programs with opportunities for partnership and investment. To learn more, visit [www.sangamo.com](http://www.sangamo.com) and connect with us on [LinkedIn](#) and [Twitter/X](#).

## **Sangamo Forward Looking Statements**

This press release contains forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to Sangamo's technologies, the presentation of data from various therapeutic and research programs and the potential of these programs to demonstrate therapeutic benefit and transform the lives of patients. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the research and development process, including the results of clinical trials; the regulatory approval process for product candidates; and the potential for technological developments that obviate technologies used by Sangamo. 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 our Securities and Exchange Commission filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2023. 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.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20240422796174/en/): <https://www.businesswire.com/news/home/20240422796174/en/>

Investor Relations & Media Inquiries

Louise Wilkie

[ir@sangamo.com](mailto:ir@sangamo.com)

[media@sangamo.com](mailto:media@sangamo.com)

Source: Sangamo Therapeutics, Inc.