



Sangamo Therapeutics to Present Neurology Pipeline Advances at the 28th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

April 28, 2025

- *One abstract accepted as platform presentation in prestigious Presidential Symposium, demonstrating potent combination of epigenetic regulation and capsid delivery technology in treating prion disease in animal models*

RICHMOND, Calif.--(BUSINESS WIRE)--Apr. 28, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the American Society of Gene & Cell Therapy (ASGCT) has accepted nine Sangamo abstracts for presentation at the 28th ASGCT Annual Meeting being held May 13-17, 2025, in-person in New Orleans, LA and in a virtual format. Presentations will highlight the progression of Sangamo's neurology pipeline, including advances in zinc finger epigenetic regulation, the latest innovations in capsid delivery engineering, and developments in modular integrase technology.

"We are proud to be showcasing our latest pipeline advances at ASGCT, including three platform presentations, reflecting our mission to develop innovative neurology genomic medicines to treat debilitating neurological diseases," said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. "We are particularly excited to be presenting, in the prestigious Presidential Symposium, our potent combination of epigenetic regulation and capsid delivery capabilities as an anticipated one-time intravenous treatment for prion disease. It will be an honor to highlight the groundbreaking potential of our work and the dedication of our teams in advancing treatment options for neurology patients in need."

Data presentations at the ASGCT Annual Meeting include three oral presentations that detail Sangamo's advances in the application of zinc finger repressors (ZFRs) as a novel class of epigenetic regulation for neurological disease targets. Two presentations – including one taking place in the Presidential Symposium – showcase how Sangamo is combining its ZFR targeting the prion gene with STAC-BBB, its intravenously administered neurotropic capsid, for the treatment of prion disease, a fatal and incurable neurodegenerative disease. These presentations describe the profound survival benefits of the treatment in disease mouse models, and the sustained, brain-wide suppression of prion protein expression in both mouse and nonhuman primate models, supporting its potential as a one-time therapeutic approach for prion disease. The third presentation focuses on the advancement of ST-503, a ZFR targeting the gene encoding Nav1.7, for the treatment of intractable, chronic neuropathic pain following intrathecal delivery.

Additional poster presentations at the ASGCT Annual Meeting will showcase advances in novel adeno-associated virus (AAV) capsid engineering and manufacturing for central nervous system (CNS) delivery. Topics range from second-generation STAC-BBB variants and receptor-targeted AAVs, to innovations in manufacturing and quality control, including strategies to enhance yield, purity, and stability, and improve analytical assessment of AAV products. Sangamo will also present updated data from its protein-guided MINT platform as an approach to enable engineering of large gene-sized pieces of DNA.

Collectively, these abstracts highlight the versatility, durability, and translational potential of ZFR-based gene regulation across diverse neurological applications.

ASGCT Annual Meeting Presentations and Invited Sessions

Neurology Epigenetic Regulation

- Sustained Brain-wide Reduction of Prion via Zinc Finger Repressors in Mice and Nonhuman Primates as a Potential One-Time Treatment for Prion Disease
 - Abstract No. 2
 - Oral Presentation – May 14; 11:30-11:45 am CT
 - Session Title: Presidential Symposium
- Preclinical Development of an AAV-delivered Zinc Finger Transcriptional Repressor Targeting the Prion Gene as a Novel Epigenetic Gene Therapy for Prion Disease
 - Abstract No. 389
 - Oral Presentation – May 17; 8:45-9:00 am CT
 - Session Title: Pharmacology/Toxicology Studies and Analytics/Assay Development Session II
- AAV-Mediated Delivery of an Engineered Zinc Finger Lead to Selective and Potent Repression of Nav1.7 in Human Sensory Neurons and Nonhuman Primates DRG Nociceptors Following Intrathecal Injection
 - Abstract No. 369
 - Oral Presentation – May 17; 8:45-9:00 am CT
 - Session Title: Translational Approaches: Gene Therapy of Neurological Diseases in Large Animal Models

AAV Engineering and Production for the Central Nervous System

- Fitness Maturation of STAC-BBB Yields Second-Generation Capsid Variants with Enhanced Delivery to the Central

Nervous System

- Abstract No. 1909
- Poster Presentation – May 15; 5:30-7:00 pm CT

- Characterization of Receptor-Targeted Blood-Brain Barrier Penetrant AAV Capsids
 - Abstract No. 1896
 - Poster Presentation – May 15; 5:30-7:00 pm CT

- The Impact of Empty Capsids on AAV Manufacturing and Strategies for Enhancing Yield, Purity, and Stability in the Production of a Novel Blood-Brain Barrier Penetrant AAV Capsid
 - Abstract No. 1464
 - Poster Presentation – May 14; 5:30-7:00 pm CT

- Recombinant Adeno-Associated Virus (rAAV) Production in Spodoptera Frugiperda (Sf9) Cells: Viral Cathepsin Mediated Capsid Cleavage and Mitigation Strategies
 - Abstract No. 987
 - Poster Presentation – May 13; 6:00-7:30 pm CT

- Assessment of Adeno-Associated Virus (AAV) Purity by Capillary Electrophoresis-Based Western
 - Abstract No. 1814
 - Poster Presentation – May 15; 5:30-7:00 pm CT

Next-Generation Genome Engineering

- A Protein-Guided Modular Integrase (MINT) Platform Enables Compact Therapeutic Payloads and Efficient Targeted Integration in T Cells
 - Abstract No. 648
 - Poster Presentation – May 13; 6:00-7:30 pm CT

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 believes that its zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and that its capsid discovery platform can expand 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 and connect with us on [LinkedIn](#) and [X](#).

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, 2024. 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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