

Encoded Therapeutics Presents Promising New Data for CNS Gene Therapy Programs at the ASGCT 28th Annual Meeting

May 13, 2025

- *AAV9-based Angelman Syndrome candidate, ETX201, demonstrates safety and target engagement in non-human primates (NHPs), with unsilencing of UBE3A across disease-relevant brain regions*
- *Multiple neurodegeneration candidates in Alzheimer's disease / tauopathies demonstrate bulk knockdown of MAPT in cortical and hippocampal brain regions of NHPs with AAV9-based intracerebroventricular delivery*
- *Vectorized miRNA candidates for chronic pain demonstrate potent and selective knockdown of Scn9a and analgesic effect in a well-established rat model*

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics Inc., a clinical-stage biotechnology company developing genetic medicines for severe central nervous system (CNS) disorders, today announced the results of preclinical studies that underscore the strength of the Company's vector engineering platform and demonstrate continued progress of programs for Angelman syndrome, Alzheimer's disease / tauopathies and chronic pain. Three poster presentations are being highlighted at the 28th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) in New Orleans, LA, May 13 – 17, 2025.

"The data we will present at ASGCT underscore the power and versatility of Encoded's vector engineering platform to target a wide array of validated disease pathways with potential one-time treatments," said Stephanie Tagliatela, co-founder and Chief Scientific Officer at Encoded. "We look forward to sharing these promising results as we advance our maturing pipeline of CNS programs."

Poster Presentation Details

ETX201: An AAV9-based Vectorized miRNA Therapeutic Candidate for Angelman Syndrome. Poster # 547. Presenter, Sirika Pillay, PhD

Date & Time: Tuesday, May 13, 6 – 7:30 PM CT

Location: Poster Hall I2

Recent data further support the potential for a one-time vectorized miRNA therapy for Angelman syndrome. The current findings demonstrate potent, dose-dependent, and selective target engagement, with gene-specific knockdown of *UBE3A-ATS* and paternal *UBE3A* upregulation in a genome-wide transcriptomic analysis. ETX201 distributes widely throughout the brain in NHPs, with ETX201 miRNA transcript present in CSF and serum. In addition, ETX201 exhibits dose-dependent upregulation of paternal *UBE3A* across multiple disease-relevant brain regions, translating to an approximate 20 percent increase in total *UBE3A* RNA, as well as increased protein levels.

Potent and Specific AAV9-miRNA Candidates Demonstrate Robust Reduction of Microtubule-associated Protein Tau (*MAPT*) in Non-Human Primates. Poster # 1440. Presenter, Veda Tatavarty, PhD

Date & Time: Wednesday, May 14, 5:30 – 7 PM CT

Location: Poster Hall I2

The first data from NHP studies of Encoded's vectorized miRNA cassettes show *MAPT* knockdown in key disease-relevant brain regions following AAV9-based delivery, including: up to 34 percent knockdown of bulk *MAPT* transcript in the cortex, 32 percent in the hippocampus, and up to 24 percent reduction in tau protein levels in the hippocampus. Consistent with these findings, our vectorized miRNA cassettes show high on-target specificity and processing fidelity and demonstrate target engagement in adult humanized tau (hTau) mice, including greater than 50 percent knockdown of *MAPT* transcript and human tau protein using an IV-delivered tool capsid. Together, these data demonstrate potent and selective knockdown of *MAPT* and highlight the potential to achieve enhanced target engagement by pairing Encoded's vectorized miRNA cassettes with novel capsids designed for superior biodistribution in the brain.

Developing an AAV-based Gene Therapy for Chronic Pain Through Identification of Potent and Selective Artificial miRNA Candidates to Knockdown *SCN9A*. Poster # 1925. Presenter, Chao Tai, PhD

Date & Time: Thursday, May 15, 5:30 – 7 PM CT

Location: Poster Hall I2

New data in chronic pain include the first demonstration of preclinical efficacy for Encoded's *SCN9A*-targeted vectorized miRNA candidates in a rat pain model, showing a durable analgesic effect and up to 70 percent *Scn9a* knockdown in DRG tissue. These data on our *SCN9A*-targeting miRNA candidates confirm potent and dose-dependent knockdown observed in vitro, with transcriptome-wide selectivity and favorable miRNA processing characteristics.

About Encoded Therapeutics

Encoded Therapeutics is a clinical-stage genetic medicines company developing potentially one-time, disease-modifying therapies for severe CNS disorders. Our proprietary vector engineering technology combines novel regulatory elements and payloads with AAV vectors, unlocking innovative solutions for intractable CNS conditions. Our lead clinical-stage program, ETX101 for Dravet syndrome, targets the underlying cause of the disorder through selective upregulation of *SCN1A* for potentially long-lasting benefit. Encoded's second program, ETX201, is a development-stage vectorized miRNA-based gene therapy designed to restore expression of *UBE3A* in individuals with Angelman syndrome. In parallel, we are advancing potentially best-in-class programs for common CNS conditions, including chronic pain and Alzheimer's disease / tauopathies. Harnessing our proprietary technology platform and expertise, we can efficiently advance programs from discovery through clinical development. Encoded is committed to pioneering breakthrough treatments for CNS disorders. For more information, please visit www.encoded.com.

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