

Encoded Therapeutics Highlights Progress in Dravet Syndrome Program and Pipeline Ahead of Company Presentation at 44th Annual J.P. Morgan Healthcare Conference

January 08, 2026

- *Positive interim efficacy data in 2025 underscore potential of ETX101 as a one-time, disease-modifying medicine for Dravet syndrome –*
- *Internal GMP manufacturing capable of supporting pivotal trial and commercialization of ETX101 and gene therapy pipeline –*
- *CEO Kartik Ramamoorthi, Ph.D. to present corporate overview at J.P. Morgan Healthcare Conference –*

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics Inc. (“Encoded”), a clinical-stage biotechnology company developing genetic medicines for severe neurological disorders, today provided an update on the significant progress made during 2025 with its Dravet syndrome candidate, ETX101, including an overview of the positive interim Phase 1/2 clinical results announced last month, establishment of GMP manufacturing capabilities to support its pipeline of gene therapies, and progress across its overall pipeline.

“Last year marked a period of significant execution for Encoded, including meaningful progress across our pipeline and manufacturing capabilities. Data that were recently presented at the American Epilepsy Society Annual Meeting underscore the potential clinical impact of ETX101 for people with Dravet syndrome. With our highly experienced team and an operational GMP manufacturing facility we are well-positioned to initiate a pivotal trial of ETX101 in 2026,” said Kartik Ramamoorthi, Ph.D., Chief Executive Officer of Encoded Therapeutics. “We are excited about multiple near-term catalysts in the year ahead and are focused on executing our vision to deliver transformative gene therapies to patients.”

Progress Across Dravet Syndrome Program

The Company’s clinical development of ETX101 reached a strategic inflection point, marked by significant progress across the following clinical and manufacturing milestones:

- ETX101 demonstrated dose-dependent and sustained reductions in monthly countable seizure frequency. Progressive and clinically meaningful gains were observed in multiple measures of neurodevelopment across initial dose levels, supporting the potential for ETX101 to prevent neurodevelopmental stagnation with early intervention.
- Attained Regenerative Medicine Advanced Therapy (RMAT) designation for ETX101 following review of preliminary clinical data from POLARIS Phase 1/2 studies.
- Launched a fully operational GMP manufacturing facility, successfully producing clinical material to supply the pivotal study while establishing a foundation that is readily scalable to support future commercial demand. The facility’s integrated CMC and GMP production capabilities enable tight control over quality and timelines that de-risk supply and preserve economic flexibility.

- Strengthened clinical leadership team with the addition of Joseph Sullivan, M.D., as Vice President of Clinical Development.

Progress Across Preclinical Pipeline

Preclinical programs further advanced toward clinical development with robust proof-of-concept data in non-human primates (NHPs) presented at key scientific meetings:

- **Chronic pain:** Durable knockdown of *SCN9A* ($\text{Na}_v1.7$) following one-time intrathecal lumbar administration of AAV9-based miRNA candidate presented at the European Society of Gene & Cell Therapy Annual Congress. Level of target engagement observed in NHPs exceeded the threshold for pain relief established in rodent chronic pain models.
- **Angelman syndrome:** Strong safety and target engagement data with unsilencing of *UBE3A* across disease relevant brain regions for AAV9-based candidate ETX201 presented at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, and for IV-delivered novel capsid candidate at the Foundation for Angelman Therapeutics (FAST) Global Science Summit.
- **Tauopathies:** Potent and robust knockdown of MAPT (tau) with AAV9 candidates presented at the ASGCT Annual Meeting.

2026 Anticipated Milestones

The Company expects important milestones in 2026 to reflect ongoing program progress related to:

- Interim efficacy results from the highest dose level of the POLARIS clinical program;
- Initiation of ETX101 pivotal study; and
- Additional progress across the early-stage pipeline, including nomination of a development candidate for chronic pain.

J.P. Morgan Healthcare Conference Presentation Details

Dr. Ramamoorthi will present a corporate overview and 2026 outlook on Monday, January 12, 2026, starting at 5:30 p.m. PT at The Westin St. Francis Hotel (Golden Gate Room, 32nd Floor).

About ETX101

ETX101 is a potential one-time, disease-modifying AAV9-based gene regulation therapy for *SCN1A*+ Dravet syndrome. ETX101 is designed to target the underlying cause of Dravet syndrome by increasing *SCN1A* expression in GABAergic inhibitory neurons. By addressing this root mechanism, ETX101 has the potential to treat the full spectrum of Dravet syndrome symptoms, including seizures, communication and cognitive impairment, behavioral issues, and motor dysfunction. ETX101 has received Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug Designations from the FDA, as well as Orphan Designation from the EMA.

About the POLARIS Program

POLARIS is a comprehensive clinical investigation of ETX101 in children with *SCN1A*+ Dravet syndrome, comprising multiple Phase 1-3 clinical trials. The first phase of POLARIS comprises three open-label, Phase 1/2 dose-escalation, multi-center trials (ENDEAVOR Part 1 (US), EXPEDITION (UK), and WAYFINDER (Australia)) in infants and young children 6 months to <7 years of age. The primary objective of the studies is to assess the safety and tolerability of ETX101. Secondary objectives are to evaluate the preliminary efficacy of ETX101 by assessing the percent change from baseline in monthly countable seizure frequency (MCSF), as well as the impact on the neurodevelopmental symptoms of Dravet syndrome, including cognition, communication, and motor function.

About Encoded Therapeutics

Encoded Therapeutics is a clinical-stage genetic medicines company developing one-time therapies for severe neurological disorders. Our vector engineering platform enables highly selective, potent, and durable gene modulation. The lead program, ETX101, is designed to address the underlying cause of Dravet syndrome by durably upregulating *SCN1A* and is currently in Phase 1/2 clinical trials. Building on this foundation, we are advancing programs to modulate validated genetic targets in chronic pain, Angelman syndrome, and Alzheimer's disease/tauopathies. With integrated discovery, development, and manufacturing capabilities, we are positioned to efficiently move programs from concept through the clinic. We are driven by a mission to meaningfully improve the lives of patients and families affected by devastating neurological disorders. For more information, please visit www.encoded.com.

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