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# Encoded Therapeutics Presents New Clinical Data from POLARIS Phase 1/2 Trials of ETX101 Gene Therapy in Dravet Syndrome at the ASGCT 2026 Presidential Symposium

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*– ETX101 demonstrated robust and durable seizure reductions through 52 weeks after a single dose –*

*– Clinically-meaningful improvements in multiple adaptive behavior domains were observed, demonstrating measurable gains in daily functioning –*

*– ETX101 demonstrated the potential to rescue developmental stagnation in the youngest treated children, with 52-week cognitive trajectories within the neurotypical range –*

*– Favorable safety profile across all four dose levels, with no treatment related serious adverse events –*

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics, Inc. (“Encoded”), a clinical stage biotechnology company developing precision genetic medicines for severe neurological disorders, today will present an expanded dataset from the ongoing POLARIS Phase 1/2 trials of ETX101, its investigational AAV9 based gene regulation therapy designed as a one time, disease modifying treatment for *SCN1A*+ Dravet syndrome. The update includes additional patients, early data from the top dose level (DL4), and longer term outcomes that further define the emerging clinical profile of ETX101 in children aged 6 months to 7 years. These results will be featured in an oral presentation during the Presidential Symposium (Wednesday, May 13, 2:28-2:39 p.m. ET) at the 29th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT).

Treatment with a single dose of ETX101 resulted in a robust and dose-dependent antiseizure effect, with durability through 52 weeks of observation. Clinically meaningful improvements in adaptive behavior were reported across the full age range tested. Notably, children treated before age 2 rapidly diverged from the developmental stagnation expected from natural history, with cognitive trajectories generally consistent with neurotypical development.

**“**Parents of children with Dravet syndrome live with the fear of every seizure and the heartbreak of watching development stall,” said Mary Anne Meskis, CEO of the Dravet Syndrome Foundation. “To see the early and robust seizure reductions paired with meaningful developmental gains is profoundly encouraging. Families have been waiting for therapies that don’t just manage symptoms but give their children a chance to keep learning and growing.”

## ASGCT Oral Presentation Highlights

*All analyses reflect data through the April 10, 2026, data cutoff.*

**Seizure Reduction:**

- From Week 5 through Week 52, durable, dose-dependent antiseizure effects were observed with approximately a 76% median monthly countable seizure frequency (MCSF) reduction at DL3 (n=3) – during a developmental window typically associated with increasing seizure

burden despite treatment with standard-of-care antiseizure medicines.
- Early data from DL4 demonstrated continued dose-dependent antiseizure activity, with the strongest response observed in patients who did not receive sirolimus (n=4), consistent with molecular and animal data showing that sirolimus dampens the therapeutic signal by reducing protein expression. No differences in safety outcomes were observed between patients that received sirolimus and those who did not.

**Neurodevelopmental Improvements:**

- Patients who reached 52 weeks of observation (n=9) demonstrated improvements in multiple domains of adaptive behavior based on the caregiver interview-based Vineland Adaptive Behavior Scales (VABS 3). The most notable gains were observed in receptive and expressive communication and motor function, with meaningful progress also observed in self care and social interaction.
- In patients treated before age 2, progressive gains in cognition were evident as early as Week 16 (n=11) and continued through Week 52 (n=4), as measured by the Bayley Scales of Infant and Toddler Development (Bayley 4). These data show that these young patients rapidly diverged from the stagnation seen in the ENVISION natural history study, with trajectories generally consistent with neurotypical development over the 52-week observation period.

“Watching these young children not only achieve durable seizure reduction but also show early evidence of neurodevelopmental rescue is truly remarkable,” said Sal Rico, M.D., Ph.D., Chief Medical Officer of Encoded. “These data reinforce our belief that ETX101 has the potential to change the course of the disease and future outlook for the Dravet community.”

To date, ETX101 has shown a favorable safety profile and has been well-tolerated across all four dose levels, with no treatment- or procedure-related serious adverse events. The most common treatment-related adverse events were transaminase elevations (a known AAV class effect), which were clinically asymptomatic and resolved in all participants.

## About the POLARIS Clinical Development Program

The POLARIS program is a comprehensive clinical investigation of ETX101 in children and adolescents with *SCN1A*+ Dravet syndrome, comprising multiple Phase 1-3 clinical trials. The first phase of POLARIS includes three ongoing open-label, Phase 1/2 dose-escalation, multicenter trials (ENDEAVOR Part 1 (US), EXPEDITION (UK), and WAYFINDER (Australia)) in infants and young children aged 6 months to 7 years. ENDEAVOR Part 1B, an expansion study in the US, is actively enrolling children and adolescents aged 4 to 18 years. These studies are evaluating the safety and preliminary efficacy of ETX101 in children and adolescents with Dravet syndrome due to variants in the *SCN1A* gene. The pivotal ENDEAVOR Part 2 study is also ongoing, evaluating seizure and neurodevelopmental outcomes in young children aged 6 months to 4 years.

## About ETX101

ETX101 is an investigational AAV9-based gene regulation therapy designed to increase the expression of the *SCN1A* gene to restore sodium channel function in inhibitory interneurons. By targeting the 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. The therapy is administered via a single intracerebroventricular (ICV) injection and is designed for long-term benefit. ETX101 has received Breakthrough Therapy, Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug designations from the FDA. It also was selected for the FDA's CMC Development and Readiness Pilot (CDRP) Program and received Orphan designation from the European Medicines Agency (EMA).

## About Encoded Therapeutics

Encoded Therapeutics is a clinical-stage biotechnology company developing one-time precision genetic medicines for severe monogenic and common neurological disorders. The company's vector engineering platform enables highly targeted and cell-type-selective control of gene expression in the brain and peripheral nervous system, allowing potent and precise modulation of disease-relevant genes to address underlying disease biology. Encoded's end to end innovation engine-spanning discovery, development, and in house GMP manufacturing-creates a streamlined path to advance a diversified pipeline of one time treatments across a broad range of neurological conditions. Encoded is 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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