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Encoded Therapeutics Announces FDA Alignment, Initiation of Pivotal Study, and ASGCT Presidential Symposium Presentation of ETX101 in Dravet Syndrome

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- *FDA alignment achieved on pivotal study design supporting a planned Biologics License Application (BLA) submission*
- *30-patient pivotal study initiated, evaluating seizure reduction and neurodevelopmental outcomes in children aged 6 months to <4 years*
- *Expansion of open label study is ongoing in patients aged 4 to <18 years*
- *ETX101 selected for Presidential Symposium presentation at the 2026 American Society of Gene & Cell Therapy (ASGCT) Annual Meeting*

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics, Inc. (“Encoded”), a clinical-stage biotechnology company developing precision genetic medicines for severe neurological disorders, today announced the successful completion of its Initial Comprehensive Multidisciplinary Regenerative Medicine Advanced Therapy (RMAT) meeting with the U.S. Food and Drug Administration (FDA), and alignment on the pivotal study design supporting the company’s planned BLA submission for ETX101, an AAV9-based gene regulation therapy designed as a one-time, disease-modifying treatment for *SCN1A*+ Dravet syndrome.

Key meeting outcomes included:

- **Confirmation of the pivotal study design**; ENDEAVOR Part 2 will evaluate a single administration of ETX101 over 52 weeks compared with a sham delayed-treatment control in 30 infants and young children aged 6 months to <4 years with *SCN1A*+ Dravet syndrome. The primary endpoint is reduction in monthly countable seizure frequency (MCSF), with a key secondary endpoint assessing improvement in cognition as measured by the Bayley Scales of Infant and Toddler Development, Fourth Edition (Bayley-4).
- **Alignment on expansion of Phase 1/2 open label study**; ENDEAVOR Part 1B will evaluate ETX101 in older participants aged 4 years to <18 years with *SCN1A*+ Dravet syndrome (n=5). The primary objective is to assess safety and tolerability. Secondary objectives are to evaluate preliminary efficacy, including reduction in MCSF and impact on neurodevelopment relative to baseline and natural history controls.

“Our alignment with the FDA on the pivotal study design marks an important step toward regulatory approval of ETX101 for children with Dravet syndrome,” said Sal Rico, M.D., Ph.D., Chief Medical Officer of Encoded. “This study design provides a rigorous and efficient path to evaluate ETX101 in the youngest population where we have the greatest opportunity to demonstrate a rapid and transformative benefit, while the ENDEAVOR Part 1B study enables the assessment of disease-modifying potential of ETX101 across the entire population of older children and adolescents with Dravet syndrome.”

Dr. Rico added, “We are proud to also announce that initiation of both ENDEAVOR Part 1B and Part 2 is already underway, demonstrating our ability to rapidly translate regulatory feedback into clinical execution. We expect patient dosing in both studies to begin in 2Q26, marking another meaningful advance in our ETX101 program and continuing our work on behalf of the Dravet

community."

In parallel with this progress, ETX101 clinical data will be presented in the Presidential Symposium at the 2026 ASGCT Annual Meeting taking place in Boston, May 11 – 15, 2026. This recognition underscores the strength of the emerging clinical data and the potential for ETX101 to meaningfully impact the course of disease in children with Dravet syndrome.

For ENDEAVOR Part 1B, initial data is expected in the fourth quarter of 2026. For ENDEAVOR Part 2, the Company plans to complete enrollment by the end of 2026 with initial data expected by the end of 2027.

Clinical Data from the POLARIS Phase 1/2 Study Support the Pivotal Study Design

In December 2025, Encoded reported positive interim data from the POLARIS Phase 1/2 program, showing significant and durable seizure reductions and neurodevelopmental gains in participants treated with ETX101. These data support the potential for ETX101 to rescue disease stagnation in young children, and together with the favorable safety profile observed to date, informed the design of the pivotal study.

ENDEAVOR Part 2 Study Design

The pivotal study is a randomized, double-blind, sham delayed-treatment controlled trial in 30 patients with *SCN1A*+ Dravet syndrome aged 6 months to <4 years. Participants will receive a single intracerebroventricular administration of ETX101. The primary endpoint of the study is percent change from baseline in MCSF at 52 weeks in patients receiving ETX101 compared with patients undergoing a sham procedure. The key secondary endpoint is change from baseline in the Bayley-4 Cognitive subdomain raw score. Additional endpoints include safety and improvements in behavior and cognition as measured by the Vineland Adaptive Behavior Scales, Third Edition (Vineland-3). ENDEAVOR Part 2 will be conducted in the United States, United Kingdom and Australia.

ENDEAVOR Part 1B Study Design

Part 1B is an open-label expansion of ENDEAVOR Part 1 to evaluate ETX101 in older children and adolescents aged 4 years to <18 years with *SCN1A*+ Dravet syndrome. The primary objective of the study is to assess the safety and tolerability of ETX101. Secondary objectives are to evaluate preliminary efficacy of ETX101 by assessing the percent change from baseline in MCSF, as well as the impact on the neurodevelopmental symptoms of Dravet syndrome, including cognition, communication, and motor function. ENDEAVOR Part 1B will be conducted in the United States.

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, as well as Orphan designation from the European Medicines Agency (EMA).

About Encoded Therapeutics

Encoded Therapeutics is a clinical-stage biotechnology company developing precision genetic medicines to transform the lives of patients with severe neurological disorders. The company's vector engineering platform enables potent and cell-type-selective regulation of gene expression, allowing for targeted modulation of disease-relevant genes. Encoded is advancing a pipeline of programs across genetic epilepsies and other neurological disorders with significant unmet need. With integrated discovery, development, and manufacturing capabilities, Encoded is positioned to efficiently move programs from concept through the clinic. 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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