ENCODED THERAPEUTICS, INC.
341 Oyster Point Blvd. South San Francisco, CA 94080

Encoded Therapeutics Announces Regenerative Medicine Advanced Therapy (RMAT) Designation Granted by U.S. FDA for ETX101 in SCN1A+ Dravet Syndrome

- Designation based on preliminary clinical evidence indicating the potential for ETX101 to address unmet medical needs associated with SCN1A+ Dravet syndrome
- Completion of enrollment in POLARIS dose-escalation clinical studies and interim clinical efficacy data presentation planned for 4Q25
- State-of-the art internal GMP facility now fully operational to support clinical and future commercial production for ETX101 and pipeline

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics Inc., a clinical-stage biotechnology company developing genetic medicines for severe neurological disorders, today announced the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to ETX101 following review of preliminary seizure data from patients treated in its ongoing Phase 1/2 program. ETX101 is an AAV9-based gene regulation therapy designed to increase *SCN1A* expression in GABAergic inhibitory neurons for the treatment of SCN1A+ Dravet syndrome. ETX101 has Fast Track, Rare Pediatric, and Orphan Drug Designations from FDA and Orphan Designation from EMA.

Additionally, the company reported that its North Carolina GMP manufacturing facility has initiated production of ETX101 to support pivotal trials expected in 1H26.

“The FDA’s decision to grant RMAT designation highlights the promising clinical efficacy and safety profile we have observed to date,” said Kartik Ramamoorthi, PhD, Chief Executive Officer of Encoded Therapeutics. “This milestone, together with the transition to in-house GMP manufacturing, underscores both the clinical potential of ETX101 and our readiness to efficiently advance the program through development. We look forward to sharing interim efficacy data from POLARIS later this year.”

RMAT designation

RMAT is an expedited program to facilitate development and review of regenerative medicine therapies that are intended to treat, modify, reverse, or cure a serious or life-threatening disease. The RMAT designation provides Encoded with early, close and frequent interactions with the FDA, organizational commitments from senior managers and other opportunities to expedite review of ETX101.

Clinical program

The POLARIS clinical development plan comprises a suite of ongoing open-label, dose-escalation clinical trials of ETX101 in infants and young children (6 months to <7 years of age) with SCN1A+ Dravet syndrome across the US, UK and Australia (ENDEAVOR Part 1, EXPEDITION, and WAYFINDER, respectively). These studies are assessing safety, tolerability, and preliminary efficacy of ETX101. Enrollment in the dose-escalation studies is expected to complete in 4Q25.

GMP manufacturing facility

Encoded's state-of-the art GMP facility in North Carolina was completed in 1Q25. Designed to support the production of multiple gene therapies, the facility enhances Encoded's strategic capabilities from early process development through commercial GMP manufacturing. By bringing key CMC activities in-house, Encoded ensures greater control over product quality and has the opportunity to accelerate development timelines and commercial readiness.

About Encoded Therapeutics

Encoded Therapeutics is a clinical-stage genetic medicines company developing potentially one-time, disease-modifying therapies for severe neurological disorders. Our proprietary vector engineering technologies combine novel regulatory elements and payloads within AAV vectors to unlock innovative solutions for intractable conditions. Our lead clinical candidate, ETX101 for Dravet syndrome, is designed to target the underlying cause of the disorder through selective upregulation of *SCN1A* for potentially long-lasting benefit. Encoded's pipeline also includes 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 neurological conditions, including chronic pain and Alzheimer's disease / tauopathies. Harnessing our proprietary technology platform, GMP manufacturing capabilities, and clinical and regulatory expertise, we can efficiently advance programs from discovery through clinical development. Encoded is committed to pioneering breakthrough treatments for neurological disorders. For more information, please visit **www.encoded.com**.

About Encoded's ETX101 Clinical Development Program POLARIS

POLARIS is a suite of clinical studies including ENDEAVOR Part 1 (US) and Part 2 (Global), EXPEDITION (UK) and WAYFINDER (Australia), to assess the safety and efficacy of ETX101 in infants and young children with SCN1A+ Dravet syndrome. POLARIS is built upon Encoded's comprehensive preclinical research, and incorporates a multipronged biomarker discovery program (ELUCIDATE), patient-focused drug development initiatives (Dravet ENGAGE), and the completed natural history study (ENVISION), the largest prospective, longitudinal natural history study of Dravet patients to date.

About ETX101

ETX101 is a potential one-time, disease-modifying gene regulation therapy targeting the underlying cause of SCN1A+ Dravet syndrome. In ETX101, a transgene encoding an engineered transcription factor under the control of a cell-selective regulatory element is delivered within a clinically-validated capsid (AAV9) to upregulate, or increase, the expression of the endogenous *SCN1A* gene. This approach is expected to increase production of $Na_V1.1$ protein sodium channels in target neurons in the brain, and to restore function. By targeting the underlying mechanism of disease, ETX101 has the potential to address the full range of symptoms associated with Dravet syndrome. ETX101 is an investigational product and its clinical efficacy and safety have not yet been established.

About Dravet Syndrome

Dravet syndrome is a severe, lifelong disorder of the central nervous system that occurs in approximately 1 in 16,000 births worldwide, with the majority of cases resulting from loss-of-function variants in the *SCN1A* gene. This developmental and epileptic encephalopathy equally affects people of both sexes and all races, manifesting in a wide array of symptoms. Frequent, prolonged, and drug-resistant seizures primarily begin in the first year of life of a typically developing infant. Severe cognitive and developmental stagnation, sleep abnormalities, motor impairment and behavioral difficulties usually become clinically evident by the second or third year of a child's life. More information about Dravet syndrome can be found at www.dravetfoundation.org.

Contacts

Jennifer Gorzelany
communications@encoded.com
650-515-9695