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

Encoded Therapeutics Announces U.S. FDA Breakthrough Therapy Designation Granted to ETX101 for the Treatment of Dravet Syndrome

– Designation recognizes the potential of ETX101 to address a serious condition with high unmet need –

SOUTH SAN FRANCISCO, Calif. – Encoded Therapeutics, Inc. (“Encoded”), a clinical-stage biotechnology company developing precision genetic medicines for severe neurological disorders, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation to ETX101 for the treatment of *SCN1A*+ Dravet syndrome. This designation is in addition to Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug, and Rare Pediatric Disease Designations, all previously granted by the FDA.

“Breakthrough Therapy Designation reflects the FDA’s recognition of both the urgent need for disease-modifying Dravet syndrome treatments and the potential of ETX101 as a meaningful treatment option for this devastating disease,” said Salvador Rico, M.D., Ph.D., Chief Medical Officer of Encoded. “Data from the ongoing Phase 1/2 studies showed durable reduction of seizures and improvements in neurodevelopment following a one-time administration. We are grateful to the FDA for recognizing the potential of ETX101 and look forward to continued collaboration to bring a meaningful new treatment option to the Dravet syndrome community as soon as possible.”

Breakthrough Therapy Designation is a process designed to expedite development and review of investigational therapies that are intended to treat a serious or life-threatening condition and for which preliminary clinical evidence indicates the therapy may demonstrate substantial improvement over available therapies on one or more clinically significant endpoints. The designation provides Encoded with more frequent and intensive FDA guidance, including the involvement of senior reviewers, as well as eligibility for rolling and priority review of the marketing application.

About Dravet Syndrome

Dravet syndrome is a severe genetic developmental and epileptic encephalopathy that begins in infancy and is most commonly caused by loss-of-function variants in the *SCN1A* gene. The disorder is characterized by frequent, prolonged seizures that are often resistant to treatment, as well as significant cognitive, behavioral, and motor impairments. Individuals with Dravet syndrome face a lifelong disease burden and an increased risk of premature mortality. Current treatment options are largely symptomatic and do not address the underlying genetic cause of the disease. More information about Dravet syndrome can be found at www.dravetfoundation.org.

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 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. 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.

Contacts

Investors/Business Development
Jennifer Gorzelany
communications@encoded.com

Media
Lori Rosen
lori@redhousecomms.com