A Clinical Study to Evaluate the Safety and Efficacy of ETX101 in Infants and Children With SCN1A-Positive Dravet Syndrome

NCT05419492

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Encoded Therapeutics
Enrollment	47 participants

Key Eligibility Criteria

Inclusion (5)

- Participant must be aged between e6 months and \<36 months in Part 1A, e48 months and \<18 years in Part 1B, e6 months and \<48 months in Part 2.
- Participant must have a predicted loss of function pathogenic or likely pathogenic SCN1A variant.
- Participant must have experienced their first seizure between the ages of 3 and 15 months.
- Participant must have a clinical diagnosis of Dravet syndrome or the treating clinician must have a high clinical suspicion of a diagnosis of Dravet syndrome.
- Participant is receiving at least one prophylactic antiseizure medication.

Exclusion (8)

- Participant has another genetic mutation or clinical comorbidity which could potentially confound the typical Dravet phenotype.
- Participant has a known central nervous system structural and/or vascular abnormality (indicated by an MRI or CT scan of the brain).
- Participant has an abnormality that may interfere with CSF distribution and/or has an existing ventriculoperitoneal shunt.
- Participant has received sodium channel blockers during the Pre-Dosing Seizure Period.
- Participant has experienced seizure freedom for a period of 4 consecutive weeks within the 90-day period prior to informed consent.

... and 3 more (see full listing online)

Locations (8 total)

UCSF Benioff Children's Hospitals, San Francisco, California, United States
Nicklaus Children's Hospital, Miami, Florida, United States
Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, Illinois, United States
... and 5 more locations