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**Phase 1/2 Study of NRTX-1001 Neural Cell Therapy in Subjects with Drug-Resistant Unilateral Mesial Temporal Lobe Epilepsy**

**Grant Award Details**

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Phase 1/2 Study of NRTX-1001 Neural Cell Therapy in Subjects with Drug-Resistant Unilateral Mesial Temporal Lobe Epilepsy

**Grant Type:** Clinical Trial Stage Projects

**Grant Number:** CLIN2-13355

**Project Objective:** The objectives of this project are 1) to conduct and complete a Phase 1, first in human study of NRTX-1 in subjects with drug-resistant mesial-temporal lobe epilepsy (MTLE), 2) to demonstrate disease-modifying activity of the GMP grade clinical lots in a preclinical model, and 3) carry out activities in preparation for the next, Phase 2 study. NRTX-1 is a cellular product consisting of human embryonic stem cell (ESC)-derived inhibitory GABA-ergic interneurons.

**Investigator:**

<b>Name:</b>	Cory Nicholas
<b>Institution:</b>	Neurona Therapeutics
<b>Type:</b>	PI

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**Disease Focus:** Epilepsy, Neurological Disorders

**Human Stem Cell Use:** Embryonic Stem Cell

**Award Value:** \$7,999,999

**Status:** Active

**Grant Application Details**

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**Application Title:** Phase 1/2 Study of NRTX-1001 Neural Cell Therapy in Subjects with Drug-Resistant Unilateral Mesial Temporal Lobe Epilepsy

**Public Abstract:**            **Therapeutic Candidate or Device**

NRTX-1001 is an inhibitory neuron cell therapy derived from human embryonic stem cells.

**Indication**

Focal epilepsy; drug-resistant mesial temporal lobe epilepsy.

**Therapeutic Mechanism**

NRTX-1001 is an inhibitory neuron cell therapy derived from human embryonic stem cells (hESCs) for the prospective treatment of drug-resistant focal epilepsy (MTLE). NRTX-1001 is delivered as a single dose by intracranial injection into the seizure focus, and is intended to distribute locally, functionally integrate, and release the inhibitory neurotransmitter GABA. NRTX-1001 is expected to persist long-term following a single dose and not require repeated administration.

**Unmet Medical Need**

Current treatments for drug-resistant MTLE include surgical resection and ablation; both are tissue-destructive and can cause serious, irreversible adverse effects. There is a clear need to develop targeted, non-tissue-destructive, and long-lasting therapies that are safe and effective for MTLE.

**Project Objective**

Initiate/conduct Phase 1; Plan for Phase 2

**Major Proposed Activities**

- Initiate and conduct FIH Study of NRTX-1001 in subjects with drug-resistant MTLE (NCT05135091). Includes preclinical and technical support.
- Clinical operations supporting planning of Stage 2. Includes preclinical and technical support.

**Statement of Benefit to California:**

Epilepsy affects more than 3 million people in the United States; one-third of epilepsy patients are drug-resistant. Transplantation of inhibitory neuron cell therapy is a novel therapeutic strategy that has shown promise in preclinical studies and could potentially provide a non-tissue-destructive therapeutic option for suppressing seizures in people with drug-resistant focal epilepsy. California medical institutions will participate in the NRTX-1001 clinical trial.

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