



Brainstorm ALS Clinical Trial

University of California, Irvine | UCLA-UCI Alpha Stem Cell Clinic at UC Irvine, and the Department of Neurology

Study Title: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Multicenter Study to Evaluate Efficacy and Safety of Repeated Administrations of NurOwn® (Autologous Mesenchymal Stem Cells Secreting Neurotrophic Factors) in Participants with Amyotrophic Lateral Sclerosis (ALS)

Lead Investigator: Namita Goyal, MD

What is the study?

The purpose of this study is to find out if repeated dosing of NurOwn® (MSC-NTF cells) a type of cell derived from your own bone marrow (the stem cells-containing fluid inside your bones), is safe without causing too many side effects in people with Amyotrophic Lateral Sclerosis and whether it may decrease the rate of decline of the disease.

This research study will compare NurOwn® to placebo. The placebo is the solution which contains no NurOwn®. During this study you may get a placebo instead of NurOwn®. Placebos are used in research studies to see if the results are due to the study drug or due to other reasons.

How long will my participation last?

You will have 14 study visits, and it will take up to 12 months to complete this research study.

How can I take part in this trial?

At UC Irvine, we will enroll approximately 50 participants.

Please note this may not be a complete list of eligibility criteria.

Main Inclusion Criteria

- Between the ages of 18 and 60 years old.
- First ALS symptoms started within the last 24 months.
- Minimum requirements on an ALS function assessment and a breathing test.
- Stable dose of Riluzole, or no Riluzole at all, for at least 30 days.

- Willing to practice contraception throughout the study.
- Citizen or permanent resident of the US or Canadian citizen.

Main Exclusion Criteria

- Prior stem cell therapy of any kind.
- Use non-invasive ventilation (NIV), diaphragm pacing system, invasive ventilation (tracheostomy), or a feeding tube.
- Radicava (edaravone) within the past 30 days or plan to begin Radicava during study.

How does it work?

MSC-NTF cells are specialized cells which are made to release chemicals to support the nervous system. To do this, study doctors will remove some of your bone marrow cells, treat them in a laboratory to become MSC-NTF, and put them back into the fluid that surrounds your spinal cord. This study and cell treatment only involves cells from your body.

Where is the study conducted?

At the University of California, Irvine Medical Center located in Orange, CA.

Will I be compensated?

You will not be compensated for your participation in this research study.

You may be refunded for the following expenses that you incur, such as parking or hotel fees while you are hospitalized for cell transplantation visits.

For more information please contact: UC Irvine Alpha Stem Cell Clinic

949-824-3990 or stemcell@uci.edu