

Phase 1B/2A Study of the Safety and Tolerability of Human Neural Stem Cells for Huntington's Disease

University of California, Irvine | UC Irvine Alpha Clinic - at <https://www.ucihealth.org/clinical-trials/alpha-clinic> | NCT07451613

Study Title: Phase 1B/2A Study of the Safety and Tolerability of Human Neural Stem Cells for Huntington's Disease

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Study Sponsor: Leslie Thompson, University of California, Irvine | CIRM

What is this study?

This study is the first time that UCI-hNSC-01 is being tested in people. The purpose of this research study is to determine whether an implantation of UCI-hNSC-01 is a safe and tolerable study intervention for patients with late stage 2 or early stage 3 Huntington's Disease.

Study Duration

Participation in the study includes up to 10 study visits over a 2-year period, with additional long-term follow-up for up to 15 years. These visits may include screening, baseline, pre-op, surgery, and implantation of hNSC-01, post-op, and follow-up visits.

How does the study drug work?

UCI-hNSC-01 is an investigational cell therapy made from human neural stem cells. In this study, the cells are surgically implanted into an area of the brain affected by Huntington's disease. Researchers hope these cells may help support existing brain cells, replace some lost cells, and help repair damaged brain connections. This study is being done to evaluate the safety, tolerability, and efficacy of UCI-hNSC-01 in people with Huntington's disease.

How do I participate?

Participants must meet the following eligibility criteria. *Please note that this is not a complete list.*

Main Inclusion Criteria

- Adults age 18 to 65 (inclusive)
- Diagnosed with Huntington's Disease and confirmed by genetic testing
- Late Stage 2 or Early Stage 3 Huntington's Disease patients
- Willing to undergo blood draws, MRI and PET scans, and a spinal fluid collection (lumbar puncture)
- Willing to use contraception during the study (1 year)

- Willing to receive medications that lower immune system activity

Main Exclusion Criteria

- You have had psychosis symptoms treated with antipsychotic medication in the past 6 months
- You have had certain cancers within the past 5 years
- You have previously received gene therapy, cell therapy, or experimental brain surgery

Will I be compensated?

Participants will receive \$200 to \$400 at specified time points during the study, for a total of up to \$2,200. The study does not reimburse out-of-pocket expenses such as travel, transportation, or accommodation.

Contact UCI Alpha Clinic

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