An hESC-derived hNSC Therapeutic for Huntington's Disease

Grant Award Details

An hESC-derived hNSC Therapeutic for Huntington's Disease

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-10953

Project Objective: File an IND application for a clinical trial using ESC-derived NSC to treat patients with Huntington's disease.

Investigator:

Name:	Leslie Thompson
Institution:	University of California, Irvine
Type:	PI

Disease Focus: Huntington's Disease, Neurological Disorders

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $5,635,393

Status: Active

Grant Application Details

Application Title: An hESC-derived hNSC Therapeutic for Huntington's Disease

Public Abstract:

Therapeutic Candidate or Device

The therapeutic candidate is a human Neural Stem Cell product to prevent or delay disease symptoms for treatment of Huntington's disease (HD).

Indication

Huntington's disease, a progressive, degenerative brain disease, typically strikes in midlife with no disease modifying treatment
treatments exist.

Therapeutic Mechanism

Based on our pre-clinical studies, the human neural stem cells engraft and differentiate into neuronal populations, express the neurotrophic factor BDNF and reduce mutant Huntingtin protein accumulation. Further, host tissue forms synaptic contacts with transplanted cells and may provide new and functional connections to reduce the aberrant cortical excitability in HD. These molecular and histological improvements correlate with improvement in behavior and electrophysiological deficits.

Unmet Medical Need

No treatment currently exists that can slow or prevent the unrelenting progression of Huntington's disease, a devastating brain disease,
therefore a completely unmet medical need exists.

Project Objective

File an Investigational New Drug request with FDA.

Major Proposed Activities

- Good Manufacturing Practice (GMP) manufacturing and characterization of the cell product to supply the first in human study.
- Good laboratory practice (GLP) long term safety, biodistribution and tumorigenicity studies in HD modeled and Wt mice.
- Investigational New Drug (IND) preparation, publishing and submission .

Statement of Benefit to California:

The disability, loss of personal freedom and earning potential, and costly institutional care of HD is devastating. Developing a therapeutic product will allow patients to live independently longer after diagnosis, and result in saving considerable costs for healthcare and caregiving, and extending the quality of life for HD patients and their family members. It will also benefit California through new technologies and intellectual property resulting in possible job creation and revenues.

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