Human Embryonic Stem Cell-Derived Neural Stem Cell Transplants in Amyotrophic Lateral Sclerosis

Grant Award Details

Human Embryonic Stem Cell-Derived Neural Stem Cell Transplants in Amyotrophic Lateral Sclerosis

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-08552

Project Objective: Pre-IND meeting with the FDA

Investigator:

Name:	Lawrence Goldstein
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Amyotrophic Lateral Sclerosis, Neurological Disorders

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $1,790,000

Status: Closed

Progress Reports

Reporting Period: OM#1 - NOT ACHIEVED see checklist

View Report

Grant Application Details

Application Title: Human Embryonic Stem Cell-Derived Neural Stem Cell Transplants in Amyotrophic Lateral Sclerosis

Public Abstract:

Translational Candidate

Spinal cord injections of human embryonic stem cell (hESC)-derived allogeneic neural stem cells (heNSCs) for treatment of ALS

Area of Impact

Treatment of Amyotrophic Lateral Sclerosis (ALS)

Mechanism of Action

Although the exact molecular mechanism of action is unknown, extensive research supports the concept that the behavior of defective astrocytes is key to the death of motor neurons and the development and progression of ALS. Allogeneic neural stem cells (heNSCs) injected into the spinal cord migrate and differentiate into functional astrocytes which can protect and support endogenous neurons, preventing further motor neuron loss and disease progression.

Unmet Medical Need

ALS is a disease for which there is literally no currently effective therapy. While there are some mild palliative approaches to treatment, in virtually all cases the diagnosis of ALS is effectively equivalent to a death sentence.

Project Objective

Pre-IND meeting with the FDA

Major Proposed Activities

- Scale up manufacturing of product for proposed studies and perform product characterization, function and efficacy testing.
- Develop in vitro methods for testing product function, efficacy and safety.
- Perform pilot in vivo tests for determination of cell survival, fate, safety. Develop and standardize in vivo and in vitro tumorigenicity methods.

Statement of Benefit to California:

ALS is a disease for which there is literally no currently effective therapy. While there are some mild palliative approaches to treatment, in virtually all cases the diagnosis of ALS is effectively equivalent to a death sentence. Clearly, in view of the dire prospects facing these patients, aggressive action on multiple, parallel therapeutic fronts is critical. It is important in our view to develop an aggressive set of cell therapy programs and have multiple “shots on goal” in parallel.
