
ASCENT- Advanced Stem Cell Enteric Neuropathy Therapy

Grant Award Details

ASCENT- Advanced Stem Cell Enteric Neuropathy Therapy

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-08471

Project Objective: Successful Pre-IND meeting with the FDA

Investigator:

Name:	Tracy Grikscheit
Institution:	Children's Hospital of Los Angeles
Type:	PI

Disease Focus: Intestinal Disease, Metabolic Disorders, Neurological Disorders, Pediatrics

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$7,077,352

Status: Active

Grant Application Details

Application Title: ASCENT- Advanced Superdonor Cellular Enteric Neuropathy Therapy

Public Abstract:**Translational Candidate**

ASCENT - Advanced Superdonor Cellular Enteric Neuropathy Therapy, is a donor progenitor cell population that replaces the enteric nervous system.

Area of Impact

ASCENT would treat enteric neuropathies including Hirschsprung disease and total intestinal aganglionosis which currently have no direct therapy

Mechanism of Action

Our goal is to develop an allogeneic "off the shelf" cellular therapy to treat enteric neuropathies before surgical interventions are needed or to rescue patients in whom effects of the ENS defect persist. We propose to generate a cellular therapy from the starting material of "superdonor" human iPS cell lines. ASCENT - Advanced Superdonor Cellular Enteric Neuropathy Therapy, is a donor progenitor cell population that, after transplantation in vivo, replaces absent functional ENS components.

Unmet Medical Need

There are no direct therapies for enteric neuropathies and ASCENT would be the first cellular therapy for a broad class of severe disease including Hirschsprung disease and other enteric neuropathies that are morbid and mortal.

Project Objective

Successful Pre-IND meeting with the FDA

Major Proposed Activities

- Manufacture ASCENT to supply the proposed studies that will assess safety and efficacy
- Determine the optimal dosing of ASCENT as well as assess clinical safety
- Completion of nonclinical safety studies in order to schedule and complete a Pre-IND meeting

Statement of Benefit to California:

Enteric neuropathies cost the state of California hundreds of millions of dollars and cost the people of California more because of the severe problems including death that result from this class of diseases. This proposal benefits California in two ways: by supporting science and the industries in California that grow from ongoing investigation, but also by reducing the medical costs and suffering of patients with enteric neuropathic conditions with development of a novel and needed therapy.

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