MRI Guided Delivery of Neural Progenitor Cells Secreting GDNF for the Treatment of Parkinson's disease

Grant Award Details

MRI Guided Delivery of Neural Progenitor Cells Secreting GDNF for the Treatment of Parkinson's disease

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-11059

Project Objective: Manufacturing and IND enabling preclinical studies to support MRI Guided Delivery of Neural Progenitor Cells Secreting GDNF for the Treatment of Parkinson's disease

Investigator:

Name:	Krystof Bankiewicz
Institution:	University of California, San Francisco
Type:	PI

Disease Focus: Neurological Disorders, Parkinson's Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $5,811,340

Status: Active

Grant Application Details

Application Title: MRI Guided Delivery of Neural Progenitor Cells Secreting GDNF for the Treatment of Parkinson's disease

Public Abstract:

Therapeutic Candidate or Device

CNS10-NPC-GDNF is a neural progenitor cell line transfected with glial cell line derived neurotrophic factor (GDNF)

Indication

Mid-stage Parkinson's disease (UPDRS stage III or lower)

Therapeutic Mechanism

Degeneration of dopaminergic neurons that project from the substantia nigra to the striatum causes the primary motor symptoms of Parkinson's disease. CNS10-NPC-GDNF cells will be transplanted into the putamen, and are expected undergo limited migration to areas of degeneration, induce sprouting of dopaminergic terminals and protect dopamine cell bodies. The cells can mature into astrocytes that may provide additional protection of degenerating regions through secretion of supportive factors.

Unmet Medical Need

Current treatments provide symptomatic relief of Parkinson's disease (PD), but become less effective over time as they have no effect on the disease process. CNS10-NPC-GDNF is expected to slow the disease progression by inducing sprouting of dopaminergic terminals and protecting dopaminergic cells.

Project Objective

Complete pre-clinical studies, and file an IND.

Major Proposed Activities

- Manufacture of CNS10-NPC-GDNF to supply the proposed clinical trial
- Demonstrate longterm lack of tumorigenicity and safety in rats
- Demonstrate safety and tolerability of CNS10-NPC-GDNF in aged MPTP lesioned non-human primate model of Parkinson's disease

Statement of Benefit to California:

Parkinson's disease is a debilitating disease, which puts a huge burden on state resources through the need for care givers and medical care. While primarily an effort to reduce patient and family suffering, this project will also ease the cost of caring for PD patients in California if successful. This in turn will attract scientists, clinicians, and biotech companies to the state of California thus increasing state revenue and state prestige in this rapidly growing field.
