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

**Grant Award Details**

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

**Investigator:**

<b>Name:</b>	Krystof Bankiewicz
<b>Institution:</b>	University of California, San Francisco
<b>Type:</b>	PI

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**Disease Focus:** Neurological Disorders, Parkinson's Disease

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$5,811,340

**Status:** Active

**Grant Application Details**

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**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.

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