hESC-derived NPCs Programmed with MEF2C for Cell Transplantation in Parkinson's Disease

Grant Award Details

hESC-derived NPCs Programmed with MEF2C for Cell Transplantation in Parkinson's Disease

Grant Type: Disease Team Therapy Planning I

Grant Number: DR2-05272

Investigator:

Name:	Stuart Lipton
Institution:	Sanford Burnham Prebys Medical Discovery Institute
Type:	PI

Disease Focus: Neurological Disorders, Parkinson's Disease

Award Value: $96,448

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Grant Application Details

Application Title: hESC-derived NPCs Programmed with MEF2C for Cell Transplantation in Parkinson's Disease

Public Abstract:

We proposes to use human embryonic stem cells (hESCs) differentiated into neural progenitor/stem cells (NPCs), but modified by transiently programming the cells with the transcription factor MEF2C to drive them more specifically towards dopaminergic (DA) neurons, representing the cells lost in Parkinson's disease. We will select Parkinson's patients that no longer respond to L-DOPA and related therapy for our study, because no alternative treatment is currently available. The transplantation of cells that become DA neurons in the brain will create a population of cells that secrete dopamine, which may stop or slow the progression of the disease. In this way, moderate to severely affected Parkinson's patients will benefit.

The impact of development of a successful cell-based therapy for late-stage Parkinson's patients would be very significant. There are approximately one million people in the United States with Parkinson's disease (PD) and about ten million worldwide. Though L-DOPA therapy controls symptoms in many patients for a period of time, most reach a point where they fail to respond to this treatment. This is a very devastating time for sufferers and their families as the symptoms then become much worse. A cell-based therapy that restores production of dopamine and/or the ability to effectively use L-DOPA would greatly improve the lives of these patients. Because of our extensive preclinical experience and the clinical acumen of our Disease Team, we will be able to quickly adapt our procedures to human patients and be able to seek an IND from the FDA within four years.

Statement of Benefit to California:

It is estimated that the cost per year for a Parkinson's patient averages over $10,000 in direct costs and over $21,000 in total cost to society (in 2007 dollars). With nearly 40 million people in California and with one in 500 estimated to have Parkinson's (1.5-2% of the population over 60 years of age), there are approximately 80,000 people in California with Parkinson's disease. Thus, Parkinson's disease is a significant burden to California, not to mention the devastating effect on those who have the disease and their families. A therapy that could halt the progression or reverse Parkinson's disease would be of great benefit to the state and its residents. It would be particularly advantageous if the disease could be halted or reversed to an early stage, since the most severe symptoms and highest costs of care are associated with the late stages of the disease. Cell-based therapies offer the hope of achieving this goal.
