Neural Stem Cell-Based Therapy For Parkinson's Disease

Grant Award Details

Neural Stem Cell-Based Therapy For Parkinson's Disease

Grant Type: Disease Team Therapy Planning I

Grant Number: DR2-05431

Investigator:

Name:	Marcel Daadi
Institution:	Sanford Burnham Prebys Medical Discovery Institute
Type:	PI

Disease Focus: Neurological Disorders, Parkinson's Disease

Award Value: $63,952

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Grant Application Details

Application Title: Neural Stem Cell-Based Therapy For Parkinson's Disease

Public Abstract:

Ongoing degeneration of dopaminergic (DA) neurons in the midbrain is the hallmark of Parkinson's disease (PD), a movement disorder that manifests with tremor, bradykinesia and rigidity. One million Americans live with PD and 60,000 are diagnosed with this disease each year. Although the cost is $25 billion per year in the United States alone, existing therapies for PD are only palliative and treat the symptoms but do not address the underlying cause. Levodopa, the gold standard pharmacological treatment to restore dopamine, is compromised over time by decreased efficacy and particularly increased side effects over time. Neural transplantation is a promising strategy for improving dopaminergic dysfunction in PD. The rationale behind neural transplantation is that grafting cells that produce DA into the denervated striatum will reestablish regulated neurotransmission and restore function. Indeed, over 20 years of research using fetal mesencephalic tissue as a source of DA neurons has demonstrated the therapeutic potential of cell transplantation therapy in animal model of PD and in human patients. However, there are limitations associated with primary human fetal tissue transplantation, including high tissue variability, lack of scalability, ethical concerns and inability to obtain an epidemiologically meaningful quantity of tissue. Thus, the control of the identity, purity and potency of these cells becomes exceedingly difficult and jeopardizes both the safety of the patient and the efficacy of the therapy. Thus the search of self-renewable sources of cells is a very worthwhile goal with societal importance and commercial application.

Human neural stem cells are currently the only potential reliable and continuous source of homogenous and qualified populations of DA neurons for cell therapy for PD. Such cell source is ideal for developing a consistently safe and efficacious cellular product for treating large number of PD patients in California and throughout the world

We have developed a human neural stem cell line with midbrain dopaminergic properties and the technology to make 75% of the neuronal population express dopamine. We have also shown that these cells are efficacious in the most authentic animal model of PD. We now propose to conduct the manufacturing of these cells in conjunction with the safety and efficacy testing to bring this much needed cellular product to PD patients and treat this devastating disease.

Statement of Benefit to California:

In this grant application we propose to develop a unique technology to manufacture neurons that will be used to treat patients suffering from Parkinson's disease. One million Americans live with PD and 60,000 are diagnosed with this disease each year. Although the cost is $25 billion per year in the United States alone, existing therapies for PD are only palliative and treat the symptoms but do not address the underlying cause. Levodopa, the gold standard pharmacological treatment to restore dopamine, is compromised over time by decreased efficacy and increased side effects. Human stem cells are currently the only potential reliable and continuous source of homogenous and qualified populations of DA neurons for cell therapy for PD. Such cell source is ideal for developing a consistently safe and efficacious cellular product for treating large number of PD patients in California and throughout the world

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The CIRM grant will help us create further intellectual property pertaining to the optimization of the process of manufacturing of the cellular product we developed to treat PD. The grant will also create jobs at Californian institutions and contract companies we will work with to develop this product. Importantly, the intellectual property will be made available for licensing to biotechnology companies here in California to develop this product to treat the over 10 million people afflicted with PD world wide. Revenues from such a product will be beneficial to the California economy.