Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease

Grant Award Details

Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease

Grant Type: Tools and Technologies III

Grant Number: RT3-07800

Project Objective: To engineer and evaluate biomaterials for two separate aims; the first is scalable manufacturing of two types of hPSC-derived neurons (dopaminergic (mDA) and photoreceptor (PR)). The second objective is to identify a biomaterial to improve survival and integration of the mDA and PR neurons in an in vivo model.

Investigator:

Name:	David Schaffer
Institution:	University of California, Berkeley
Type:	PI

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Name:	Sanjay Kumar
Institution:	University of California, Berkeley
Type:	Co-PI

Disease Focus: Neurological Disorders, Parkinson's Disease, Vision Loss

Human Stem Cell Use: Embryonic Stem Cell, iPS Cell

Award Value: $1,239,276

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 3

View Report

Grant Application Details

Application Title: Engineered Biomaterials for Scalable Manufacturing and High Viability Implantation of hPSC-Derived Cells to Treat Neurodegenerative Disease

Public Abstract: Cell replacement therapies (CRTs) have considerable promise for addressing unmet medical needs, including incurable neurodegerative diseases. However, several bottlenecks hinder CRTs, especially the needs for improved cell manufacturing processes and enhanced cell survival and integration after implantation. Engineering synthetic biomaterials that present biological signals to support cell expansion, differentiation, survival, and/or integration may help overcome these bottlenecks. Our prior work has successfully generated synthetic biomaterial platforms for the long-term expansion of human pluripotent stem cells (hPSCs) at large scale, efficient differentiation of hPSCs into dopaminergic progenitors and neurons for treating Parkinson's Disease, and modulation of stem cell function to promote neuronal differentiation within the brain. We now propose to advance this work and engineer two synthetic biomaterial platforms to treat neurodegenerative disease, in particular Parkinson's Disease and Retinitis Pigmentosa. Specifically, our central goals are to further engineer biomaterial systems for scalable hPSC differentiation into dopaminergic and photoreceptor neurons, and to engineer a second biomaterial system as a biocompatible delivery vehicle to enhance the survival and engraftment of dopaminergic and photoreceptor neurons in disease models. The resulting modular, tunable platforms will have broad implications for other cell replacement therapies to treat human disease.

Statement of Benefit to California: This proposal addresses critical translational bottlenecks to stem cell therapies that are identified in the RFA, including the development of fully defined, xenobiotic free cell manufacturing systems and the development of clinically relevant technologies to enhance the survival and integration of human stem cell therapies. The proposed platform technologies for expanding and differentiating pluripotent stem cells in a scaleable, reproducible, safe, and economical manner will initially be developed for treating two major neurodegenerative disorders - Parkinson's Disease and Retinitis Pigmentosa - that affect the well-being of hundreds of thousands of Californians and Americans. In addition, the biomaterial platforms are designed to be modular, such that they can be re-tuned towards other target cells to even more broadly enable cell replacement therapies and enhance our healthcare. This work will thus strongly enhance the scientific, technological, and economic development of stem cell therapeutics in California.

Furthermore, the principal investigator has a strong record of translating basic science and engineering towards clinical development within industry, particularly within California. Finally, this collaborative project will focus research groups with many students on an important interdisciplinary project at the interface of science and engineering, thereby training future employees and contributing to the technological and economic development of California.