Clinical Translation of Autologous Regenerative Cell Therapy for Blindness

Grant Award Details

Clinical Translation of Autologous Regenerative Cell Therapy for Blindness

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-11265

Project Objective: Completion of translational activities leading to a pre-IND meeting for autologous induced pluripotent stem cell-derived retinal pigment epithelium (AiPSC-RPE), for the treatment of maculopathies.

Investigator:

Name:	Steven Schwartz
Institution:	University of California, Los Angeles
Type:	PI

Disease Focus: Age-related macular degeneration, Vision Loss

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: $5,068,026

Status: Closed

Progress Reports

Reporting Period: Final Operational Milestone #5

View Report

Grant Application Details

Application Title: Clinical Translation of Autologous Regenerative Cell Therapy for Blindness

Public Abstract:

Translational Candidate

We are studying autologous induced pluripotent stem cell-derived retinal pigment epithelium (AiPSC-RPE) cells for the treatment of maculopathies.

Area of Impact

Maculopathies (including AMD, SMD, & MMD) may be treated with AiPSC-RPE cells to replace RPE and support photoreceptors to improve vision.

Mechanism of Action

AiPSC-RPE cells replace RPE lost to disease, and support continued photoreceptor function. Transplanted AiPSC-RPE cells perform functions of the RPE layer: providing a membrane between the neurosensory retina and the choroid permeable to ions and metabolites; phagocytosis of photoreceptor outer segments; synthesis of Bruch's membrane matrix; light absorption and improving image resolution. By performing these functions, AiPSC-RPE cells support photoreceptors, improving vision.

Unmet Medical Need

Disorders affecting the macula cause loss of central vision and disability. Maculopathies affect will affect ~20M people in the US by 2020. There are no approved treatments for these conditions. Patient specific stem cell derived retinal pigment epithelium (RPE) cells provide a potential treatment.

Project Objective

Pre-IND meeting

Major Proposed Activities

- Cell therapy product generation and formulation (7 AiPSC-RPE replicates)
- Qualification of assays for manufacturing process, development and optomization of in-process and release potency tests
- Preclinical testing of safety and efficacy

Statement of Benefit to California:

About 800,000 Californians had vision related disorders in 2016, a significant subset of which are maculopathies caused by degeneration of retinal pigment epithelium cells. There are no effective treatments for most of these conditions. Development of effective therapies for multiple forms of maculopathy, supporting recovery of the damaged retina, would offer tremendous functional benefits to many residents of the State of California, and fiscal benefits from reduced long-term healthcare costs.

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