Safety and Feasibility of Cultivated Autologous Limbal Stem Cells for Limbal Stem Cell Deficiency

Grant Award Details

Safety and Feasibility of Cultivated Autologous Limbal Stem Cells for Limbal Stem Cell Deficiency

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11650

Project Objective: Conduct a phase 1 clinical trial to assess the safety and feasibility of cultivated autologous limbal stem cells for limbal stem cell deficiency.

Investigator:

Name:	Sophie Deng
Institution:	University of California, Los Angeles
Type:	PI

Disease Focus: Corneal Damage, Vision Loss

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Adult Stem Cell

Award Value: $10,301,486

Status: Active

Grant Application Details

Application Title: Safety and Feasibility of Cultivated Autologous Limbal Stem Cells for Limbal Stem Cell Deficiency

Public Abstract:

Therapeutic Candidate or Device

Autologous cultivated limbal stem cells (cLSC)

Indication

Limbal stem cell deficiency

Therapeutic Mechanism

Restoration of a normal corneal surface using cLSC might be achieved by replenishing the LSC population and/or providing trophic factors to stimulate residual LSCs.

Unmet Medical Need

Therapy using cultivated LSC, which achieves the best clinical outcomes is a standard of care in Europe. The lack of this therapy in the US is an unmet medical needs . Our therapy has the potential to safely, adequately and successfully treat LSCD and become the standard of care in the US.

Project Objective

Complete a phase I clinical trial

Major Proposed Activities

- Manufacture and transplant 15 patient-specific cLSC in 15 subjects with LSCD and provide scleral lens treatment to 5 control subjects
- Refine and validate the new diagnostic assays for LSCD and quantify LSC function after transplantation.
- Complete the 1 year follow-up in patients treated with the cLSC to assess clinical safety of the cLSC and scleral lens.

Statement of Benefit to California:

The LSCD patient population in California differs from that outside of the US and the efficacy of all currently available LSC treatments have not been demonstrated in our patient population.. Our cLSC product has the potential to treat LSCD by increasing safety in a larger population including those in California. The new diagnostic tools will increase the accuracy of LSCD diagnosis and enable LSC function assessment in patients. Our cLSC therapy will raise the standard of care for LSCD.
