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## Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy

### Grant Award Details

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Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy

**Grant Type:** Late Stage Preclinical Projects

**Grant Number:** CLIN1-08686

**Project Objective:** Conduct a phase 1 trial to assess the safety and potential efficacy of limbal stem cell therapy to regenerate a normal corneal surface (autologous product manufacturing, trial conduct and diagnostic assay development)

**Investigator:**

<b>Name:</b>	Sophie Deng
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

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**Disease Focus:** Corneal Damage, Vision Loss

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$4,244,211

**Status:** Active

### Progress Reports

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**Reporting Period:** Final Operational Milestone #4

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### Grant Application Details

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**Application Title:** Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy

**Public Abstract:****Therapeutic Candidate or Device**

cultivated patient-specific corneal epithelial stem cells (limbal stem cells, LSC)

**Indication**

Corneal blindness from inability to heal due to corneal epithelial stem cell deficiency as a result of injury

**Therapeutic Mechanism**

Limbal stem cell deficiency (LSCD) leads to inability to heal. The most desired treatment is to replace the necessary amount of the stem cell to maintain a normal, transparent corneal surface. Any remaining LSCs will be identified and biopsy from the patient to be expanded in culture. Once sufficient amount of LSCs are produced, these LSCs will then be transplanted back to patient's eye to restore a normal corneal surface.

**Unmet Medical Need**

Cultivated LSCs has been shown to be effective and a safer treatment than direct transplantation for LSCD since 1997 in Europe. This stem therapy is not available in the United States. Our therapy will be the first patient-specific stem cell therapy to treat both unilateral and bilateral LSCD.

**Project Objective**

IND filing and ready for phase 1 trial

**Major Proposed Activities**

- LSC manufacture development and certification
- Establishment of manufacture process in GMP facility
- biomarker development

**Statement of Benefit to California:**

California is the most populated state in the USA. The number of residents with LSCD may disproportionately increase as a result of multiple environmental risk factors. A safe treatment to restore vision would be an important benefit to the people of California. Our project will further benefit California through the training of new stem-cell researchers, create more jobs, and attract funding from the federal government and investment from the private sector.

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**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/regeneration-normal-corneal-surface-limbal-stem-cell-therapy>