Regeneration of a Normal Corneal Surface by Limbal Stem Cell Therapy

Grant Award Details

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:

<table>
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<tr>
<th>Name</th>
<th>Sophie Deng</th>
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<tr>
<td>Institution</td>
<td>University of California, Los Angeles</td>
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<td>Type</td>
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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

Reporting Period: Final Operational Milestone #4
View Report

Grant Application Details

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