Here we proposed to do foundational research towards developing a stem cell-based therapy for corneal diseases. We successfully completed all proposed experiments. Through Aim 1 we discovered that corneal endothelium-like cells could be generated by differentiation of human stem cells. Novel compounds were tested that were discovered to improve the cells’ morphology and function, with or without affecting cells’ proliferation and migration. Taking this data forward into Aim 2 for \textit{in vivo} experiments in a relevant animal model, we explored successfully human stem cell-to-corneal endothelial cell differentiation and transplantation, and discovered a risk of immune rejection not previously anticipated. These data suggest that for proof-of-concept studies in the future, immunosuppression may be preferred to move such cell therapy through future proof-of-concept (POC) and FDA-enabling studies.
Public Abstract:  

Research Objective

The proposed studies will determine the optimal approaches to differentiate and transplant stem cell-derived corneal endothelial cells.

Impact

These data will provide foundational proof-of-concept data that will allow the rapid advance of a cell therapy towards clinical application.

Major Proposed Activities

- Determine optimal conditions to generate human corneal endothelial cells from human stem cells, assaying both cellular and functional markers.
- Test efficacy in a rabbit model that closely mimics human injury or degeneration, examining disease-relevant functional assays.

Statement of Benefit to California:

1. Employing California’s citizens in the research funded through CIRM and thereafter as this project advances.
2. Developing a first-in-class treatment for California’s citizens with corneal diseases affecting their vision.

Source URL: https://www.cirm.ca.gov/our-progress/awards/embryonic-stem-cells-corneal-endothelial-degeneration