CALIFORNIA STATE SCIENCE FAIR
2017 PROJECT SUMMARY

Name(s)

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Project Number

J0510

Project Title

A Novel Gene Therapy Using CRIPR/Cas9 Mediated Cellular Reprogramming

Abstract

Objectives/Goals

Gene therapy shows great promise in treating many human diseases. However, one major drawback of the current technology is that it can only be directed to a particular mutation or a single gene at best. Retinitis pigmentosa (RP) is a major cause of blindness characterized by retinal rod photoreceptors degeneration. It can be caused by numerous mutations in many genes. My goal is to restore tissue architecture and visual function by switching a mutation-venerable/sensitive rod cell type to a mutation-insensitive/resistant cone cell type. This is accomplished by inactivating Nrl, a key gene controlling binary switch between rods and cones.

Methods/Materials

I designed AAV constructs to introduce CRISPR/Cas9 system into rod photoreceptor cells in the eye. I used PCR and immunohistochemistry to assess efficiency of gene editing.

Results

We show an increase in cone like cells with concomitant preservation of both cone and rod photoreceptors and visual function in a RP models by inactivation of NRL gene.

Conclusions/Discussion

Our approach shows promise of cellular reprogramming in preventing retinal degeneration and preserving vision, and points to a novel approach in treating human diseases in a gene and mutation independent manner.

Summary Statement

I used CRISPR/Cas9 mediated gene therapy to inactivate NRL gene in rod photoreceptors in the eye and achieved prevention of retinal degeneration and restoration of visual function.

Help Received

I designed and performed the in vitro gene editing assay, and helped with immunohistochemistry and with animal experiment design. I received guidance in project design from Dr. Xin Fu at UC San Diego.