Lipid Nanoparticles Mediated Delivery Of RNA Therapeutics to Trabecular Meshwork

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BRIEF DESCRIPTION

This technology represents a groundbreaking approach to treating Primary Open Angle Glaucoma by directly targeting the trabecular meshwork pathology with lipid nanoparticle-mediated delivery of gene editing tools or anti-sense oligos.

FULL DESCRIPTION

Researchers at UCI have developed lipid nanoparticles (LNPs) that target trabecular meshwork tissue. These LNP platform can deliver mRNA, protein or antisense oligos to trabecular meshwork selectively. LNPs can deliver base editor mRNA or protein along with guide RNA or antisense oligos specifically to the trabecular meshwork (TM) in mice. This approach aims to knock out the MYOC gene, a major genetic cause of glaucoma, thereby restoring normal TM function and potentially offering a one-time cure for glaucoma.

SUGGESTED USES

- Gene therapy for glaucoma and other ocular diseases with known genetic causes
- Development of precision therapeutics for a range of genetic disorders
- Research tools for studying gene function in vivo within specific tissues
- Potential platform technology for targeted delivery of a wide range of therapeutics beyond gene editing tools

ADVANTAGES

- Direct targeting of the primary genetic cause of Primary Open Angle Glaucoma (POAG)
- Use of adenine base editors (ABEs) to avoid DNA double-strand breaks and minimize off-target effects
- Novel lipid nanoparticle (LNP) delivery system ensures high tropism to TM and low immunogenicity
- Transient expression of mRNA or protein reduces risks of prolonged Cas9 activity and potential oncogenesis
- Potential to provide a one-time, permanent therapeutic solution

PATENT STATUS

Patent Pending

CONTACT

Patricia H. Chan
patricia.chan@uci.edu
tel: 949-824-6821.

OTHER INFORMATION

KEYWORDS

mRNA technology, gene editor protein delivery, gene editing, myocilin, glaucoma, lipid nanoparticles, adenine base editing, CRISPR-Cas9, trabecular meshwork, RNP, sgRNA, antisense oligos, gene therapy

CATEGORIZED AS

- **Medical**
  - Delivery Systems
  - Disease: Ophthalmology and Optometry
  - Gene Therapy
  - Therapeutics
- **Research Tools**
  - Expression System

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5270 California Avenue / Irvine,CA 92697-7700 / Tel: 949.824.2683