Safe Vector for Glaucoma Gene Therapy

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SUMMARY

UCLA researchers from the Department of Ophthalmology have developed a novel gene therapy approach to cure eye diseases such as glaucoma using naked plasmid DNA.

BACKGROUND

Glaucoma is a debilitating eye disease that can lead to blindness. One of the major symptoms is abnormal eye pressure, which is regulated by trabecular meshwork that controls the aqueous humor outflow from the eye. Gene therapy can relieve or revert some of the symptoms in eye diseases such as glaucoma. Expression of therapeutic genes in tissues such as trabecular meshwork can alter eye pressure. Conventional gene therapy delivery methods involve using viral vectors that carry the therapeutic genetic contents. However, using viruses can cause inflammation, cancers or even death. A safer alternative is using naked plasmid DNA, which is not easily incorporated into cells when delivered to human tissues.

INNOVATION

A novel gene therapy approach for eye diseases such as glaucoma has been developed to relieve symptoms such as abnormal eye pressure. Cells in the anterior eye chamber, which includes the trabecular meshwork, are in a unique location that allows them to easily take up naked plasmid DNA. Unlike blood, the aqueous humor in the anterior chamber is “immune privileged”, and it does not contain factors that compromises the integrity of naked plasmid DNA. The special physiological features of human eyes make them ideal targets for gene delivery using plasmid DNA. The convenient placements of the eye also make it easy to use additional methods such electroporation and ultrasound to increase gene delivery efficiency. Once the therapeutic genes express in the trabecular meshwork, abnormal pressure in eyes can be altered.

APPLICATIONS

- Glaucoma
- Retinal diseases
- Diseases that involve the anterior chamber of the eyes

ADVANTAGES

- Safe
- Permanent
- Efficient
- Applicable to many eye diseases
- Easy to administer

PATENT STATUS

Country	Type	Number	Dated	Case
Germany	Issued Patent	3367982	12/28/2022	2017-519
European Patent Office	Issued Patent	3367982	12/28/2022	2017-519
France	Issued Patent	3367982	12/28/2022	2017-519
United Kingdom	Issued Patent	3367982	12/28/2022	2017-519
Japan	Issued Patent	6,962,912	10/18/2021	2017-519
United States Of America	Issued Patent	10,117,776	11/06/2018	2017-519

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OTHER INFORMATION

KEYWORDS

Gene therapy, Viral vectors, Plasmid vectors, Hydrodynamic gene delivery, Electroporation

CATEGORIZED AS

- **Medical**
  - Disease: Ophthalmology and Optometry
  - Gene Therapy

RELATED CASES

2017-519-0

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