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Gene Therapy Vector for Eye Disease

Stanford researchers have discovered the first of its kind gene therapy vector to treat eye diseases of the non-pigmented ciliary epithelium cells (NPCECs). With a gene therapy vector, a more predictable and stable treatment is possible to treat glaucoma and inflammatory conditions of the eyes when utilizing specific gene targets. The Mahajan Laboratory at Stanford used tissue culture in vitro to identify a minimal DNA promotor to drive gene expression in the NPCECs. The method has shown that a BEST2 minimal promoter (specific to NPCECs gene expression) can be manipulated and linked to a sequence of gene encoding of interest in the NPCECs to treat eye disease. Primarily, NPCECs are linked to glaucoma which affects 60 million people worldwide with no cure. Current management involves delaying the progression of disease, but many people continue to lose eyesight (due to retinal ganglion cell loss) permanently even with proper therapies. NPCECs are also involved in inflammatory conditions (anterior/iridocyclitis), pseudoexfoliation syndrome, and uveal melanoma. With extended research, this technology can provide a cure and improve treatment of a vast array of eye diseases that lack effective therapy.

Stage of Development

- The gene therapy vector has been tested in vitro. Testing has shown that the BEST2 minimal promoter can be linked to a gene encoding polypeptide, a regulatory RNA sequence, a reporter gene
- Will be tested in vivo next

Applications

- **Glaucoma treatment**– current standard of care is to slow progression but does not halt disease progression. With gene therapy vector, a more predictable and stable treatment is possible
- **Other therapies** – diseases of immunogenicity such uveitis/iridocyclitis, pseudoexfoliation syndrome, and uveal melanoma

Advantages

- **First of in class approach** - Only gene therapy vector in the market to target the NPCECs
- **Unmet medical need** – Glaucoma pathogenesis is ambiguous and additional management for this disease is needed for the 60 million people it affects worldwide
- **More predictable and stable** - Gene therapy can possibly provide a cure as well as improve your body's ability to fight disease

Patents

- Published Application: WO2021061946
- Published Application: 20220362407
- Issued: 12,611,469 (USA)

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