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Novel molecules of gene therapy for glaucoma and optic neuropathies

Stanford researchers in the Goldberg lab have discovered two novel gene therapy targets for the treatment of glaucoma and other optic neuropathies. Glaucoma is the leading cause of irreversible blindness world-wide, affecting millions of adults in the United States alone. The disease is characterized by the degeneration and loss of retinal ganglion cells (RGCs), a type of neuron that functions in transmitting visual signals from the retina to the optic nerve. Current eyedrop treatments for glaucoma only slow the progression of the disease and lose effectiveness in its later stages. Overexpression of these novel gene therapy targets has been discovered to induce differentiation of RGCs and promote their axon growth *ex vivo*, offering promising avenues for the development of regenerative therapies for glaucoma and other optic neuropathies.

Applications

- Gene therapy
- Glaucoma
- Optic neuropathy

Advantages

- Potential to reverse progression of currently irreversible ophthalmic pathologies
- No existing gene therapies for glaucoma and other optic neuropathies

Patents

- Published Application: WO2022169878

- Published Application: 20240082428

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