Docket #: S23-196

Pioneering Targeted Gene Therapy for Treatment of Glaucoma and Inner Retinal Disorders

Stanford researchers have developed a pioneering gene therapy by targeting reactive astrocytes in the optic nerve head (ONH) and modulating cyclic adenosine monophosphate (cAMP) levels for targeted treatment of glaucoma and other retinal disorders.

Despite the increasing prevalence of retinal disorders like glaucoma in the US, effective targeted therapies for afflicted patients are scarce. Current treatments, such as eye drops, merely aim to slow disease progression without addressing the root cause. Despite the pivotal role of ONH astrocytes driving the pathogenesis of glaucoma and various optic neuropathies, there is a lack of therapeutic methods targeting this cell population in retinal disorders. Addressing this gap is crucial for developing effective targeted treatments for irreversible conditions like glaucoma and other retinal disorders.

Stanford researchers have filled this critical gap by pioneering a gene therapy solution targeting reactive astrocytes in the optic nerve for the treatment of glaucoma and other inner retinal disorders. These astrocytes, when injured, differentiate into either protective or harmful reactive astrocytes, which are controlled by cAMP. This invention specifically modulates the cAMP levels, either by increasing nuclear or depleting cytosolic cAMP, promoting the proliferation of protective astrocytes, and inhibiting harmful ones, ultimately aiding in retinal ganglion cell survival after optic nerve injury in glaucoma. By targeting specific reactive astrocyte populations and modulating compartmented cAMP, this invention marks a significant advancement in targeted gene therapy and holds promise for treating glaucoma and other inner retinal disorders.

Stage of Development:

Pre-clinical. Next steps involve continued testing in preclinical models of glaucoma, followed by large animal proof-of-concept and toxicology studies.

Applications

- Targeted gene therapy for glaucoma and other inner retinal disorders
- Treatment of optic neuropathies
- Prophylaxis of CNS disorders
- Gliotherapeutics.

Advantages

- No available gene therapies for glaucoma and other optic neuropathies
- No therapeutic methods that target optic nerve head astrocytes for the treatment of glaucoma and inner retinal disorders

Publications

- Evan G. Cameron, et al. (2024). " A molecular switch for neuroprotective astrocyte reactivity". *Nature* 626, 574–582 (2024).

Patents

- Published Application: WO2025007065

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