Innovations
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

Gene Therapy for Retinal Ganglion Cell Protection in Glaucoma

Product

Heat Shock Protein Gene Therapy

Indication

Glaucoma

Value Propositions

- Protects RGC from death in the setting of glaucoma
- Provides long lasting protection with a single injection

Market

- $2 Billion—US Glaucoma Therapeutic Market in 2020 (5% CAGR through 2026)

Intellectual Property

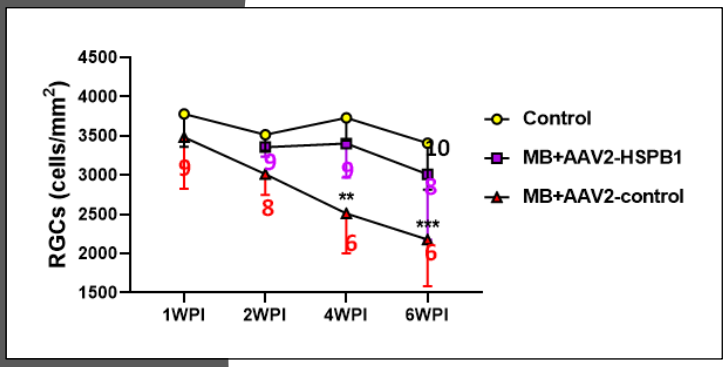
- Patent pending*
- Available for licensing

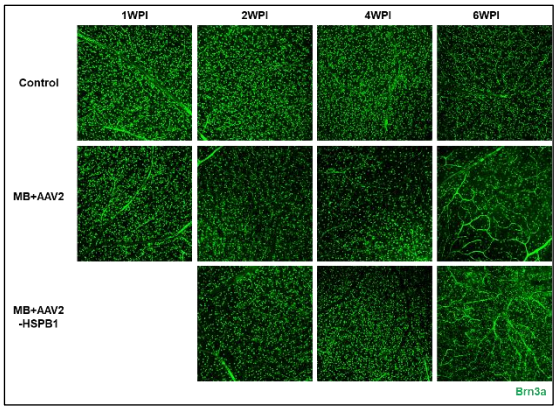
Background on CU5168H

Glaucoma is the leading cause of irreversible vision loss worldwide. All forms of glaucoma are characterized by the loss of the retinal ganglion cells (RGCs) and their axons that make up the optic nerve, which leads to optic nerve damage and visual field loss. Over 3 million people suffer from glaucoma in the US alone. Recent progress in the treatment of glaucoma has come from therapeutics that decrease IOP (intraocular pressure) to a greater extent and have a more prolonged release compared to traditional approaches. However, compliance remains a significant barrier to effective treatment for many patients, leading to damage to RGC and permanent vision loss.

Technical Innovation

Dr. Ram Nagaraj and his team at the University of Colorado have developed a gene therapy that reduces RGC death in patients with glaucoma. They constructed an AAV2 vector encoding human HSPB1 with a mini RGC-specific neurofilament light chain promoter PLE345 for specific expression in RGCs. Intravitreal injection of AAV2-HSPB1 resulted in robust expression of HSPB1 in both the soma and axons of RGCs. In mice with ocular hypertension (induced by microbead (MB) injection into the anterior chamber), a 36% decrease in RGCs (Brn3a +ve) was observed six weeks post-injection. AAV2-HSPB1 administration one week after the MB injection resulted in significant prevention of RGC loss, with only a 12% decrease at six weeks. The AAV2-HSPB1 administration also inhibited the RGC axonal defects activation of retinal microglia. The inventors believe that a single intravitreal injection can provide long-term RGC protection for those with active glaucoma or at risk for disease.





Resources & Documents:

** **Patent pending:** "Viral Vector-Based Gene Therapy For Ocular Conditions".*

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