



DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Government Owned Invention Available for License: Pigment Epithelium-Derived Factor Peptides and Their Use for Treating Retinal Degeneration

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: The National Eye Institute (NEI) seeks research co-development partners and/or licensees for the development of an AAV2-based delivery system or an eyedrop formulation to deliver a Pigment Epithelium-Derived Factor (PEDF) peptide as a gene-agnostic approach to treating inherited retinal diseases.

FOR FURTHER INFORMATION CONTACT: Inquiries related to this license opportunity should be directed to: Ricquita Pollard, Ph.D., Unit Supervisor, NCI, Technology Transfer Center, Email: ricquita.pollard@nih.gov or Phone: 240-276-5490.

SUPPLEMENTARY INFORMATION: Retinitis pigmentosa (RP) is one of the most common inherited retinal diseases (IRDs) – estimated to affect 1 in 4,000 people worldwide. Over 100,000 people in the US and 1.5 million people worldwide suffer from RP. This disease leads to progressive photoreceptor cell degeneration and, ultimately, vision loss. More than 90 genes are implicated in molecular pathways towards photoreceptor cell death. Due to this high heterogeneity, therapeutic approaches targeting specific genes generally benefit few patients. For most forms of RP, few or no medical options are available. Thus, there remains a need to identify new and more effective treatments for RP and other inherited retinal degenerations. Mutation-independent strategies to protect photoreceptors against continued damage and degradation are appealing approaches.

To delay photoreceptor degeneration, NEI proposes using neurotrophic molecules as a mutation-independent approach using PEDF. PEDF is a multifunctional member of the serine proteinase inhibitor (serpin) family with neurotrophic and antiangiogenic properties in the retina. Researchers at the NEI

developed a peptide of 17 amino acid residues (17-mer) from the receptor-binding domain of PEDF. It contains amino acid substitution at position 105 from a histidine to an alanine (PEDF 17-mer[H105A]). H105A exhibits a highly potent protective effect on photoreceptors using *in vivo* mouse models of RP.

The technology encompasses two delivery approaches for this proprietary peptide: (1) an eyedrop formulation and/or (2) an Adeno-Associated Vector 2 (AAV2). A sustained delivery system delivers the 17-mer [H105A] to treat or prevent photoreceptor degradation and vision loss in patients with IRDs (e.g., retinitis pigmentosa, Leber congenital amaurosis, cone-rod dystrophy, Stargardt-like macular degeneration or maculopathy) or age-related macular degenerations (AMD). The technology is available for licensing and co-development.

“This Notice is in accordance with 37 C.F.R. § 404.4 Authority to grant licenses.”

NIH Reference Number: E-028-2023.

Related Technologies: E-156-2023.

Product Type: Therapeutic.

Therapeutic Area(s): Eye/Ear/Nose/Throat | Ophthalmology.

Development Stage: Pre-clinical (*in vivo* validation).

Publications:

- Bernardo-Colón A, et al. H105A peptide eye drops promote photoreceptor survival in murine and human models of retinal degeneration. (PMID: 40118996)
- Valiente-Soriano FJ, et al. Pigment Epithelium-Derived Factor (PEDF) Fragments Prevent Mouse Cone Photoreceptor Cell Loss Induced by Focal Phototoxicity In Vivo. (PMID: 33008127)
- Hernández-Pinto A, et al. PEDF peptides promote photoreceptor survival in rd10 retina models. (PMID: 30980815)
- Kenealey J, et al. Small Retinoprotective Peptides Reveal a Receptor-binding Region on Pigment Epithelium-derived Factor. (PMID: 26304116).

Patens: National Phase.

Potential Commercial Applications:

- Minimally invasive therapy to prevent photoreceptor degeneration in retinal disorders IRDs and

AMD.

- RP, macular degeneration, and other related diseases.
- AAV2 vectors for delivery of genes to retinal cells both preventative and therapeutic.
- Prevent disease progression to late-stage RP.

Competitive Advantages:

- Favorable safety profile.
- Large addressable market given applications to IRDs and AMD (USD 13.7 billion in 2023 and an estimated compound annual growth rate (CAGR) of 9.6% from 2024 to 2032).
- Partially established regulatory path, as their AAV2 vector is identical to Luxturna, an FDA-approved therapy for retinal dystrophy.
- Superior diffusion and penetrability than biologics.
- Demonstrably less immunogenicity and lower production costs than biologics.
- Eye drop formulation has larger addressable market.
- Eye drop formulation provides easy administration route.
- Broad-spectrum therapeutic approach:
 - PEDF mimics the natural protective process lost in patients with inherited eye diseases.
 - Protective effects on photoreceptors are independent of the gene mutations causing degeneration.
- Chemically synthesized bioactive peptide solutions are free of inactive ingredients, causing fewer secondary effects than mixed formulations.

Collaboration Opportunity: Researchers at the NEI seek licensing and/or co-development research collaborations for the development of an AAV2-based delivery system or an eyedrop formulation to deliver a PEDF peptide as a gene-agnostic approach to treating inherited retinal diseases.

Dated: July 21, 2026.

Richard U. Rodriguez,

Associate Director,

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National Cancer Institute.

