

Emerging cross-cutting therapies for Usher syndrome

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FOUNDATION
**FIGHTING
BLINDNESS**

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Two things to keep in mind

- 1) Usher syndrome is retinitis pigmentosa with hearing loss (and sometimes vestibular issues)
- 2) RP therapies may benefit people with Usher syndrome

What is meant by “cross-cutting”?

- 1) A treatment designed to work independent of the mutated gene causing the disease
- 2) Treatment may benefit patients whether or not their gene mutation has been identified
- 3) A treatment isn't necessarily “best” because it targets the genetic cause

Foundation Fighting Blindness

What does “neuroprotective” mean?

Slows loss of photoreceptors

Helps preserve vision

Major Foundation Investments – Cross-Cutting Therapies

Nacuity (Dallas) – up to \$7.5 million

- NACA – strong antioxidant to slow vision loss (RP, others)
- Developed at Johns Hopkins
- Phase 1/2a in Australia for Usher syndrome (RP trials in 2021)

SparingVision (France) – up to €7 million

- RdCVF – rod-derived cone viability factor (protein)
- Saves cones (RP, others)
- Developed at Institut de la Vision

Major Foundation Investments – Cross-Cutting Therapies

Stephen Martin, PhD, UT Austin – \$900,000

- Neuroprotection, anti-inflammatory – slow degeneration
- Small molecule that modulates TMEM97
- Slow release formulation

W. Clay Smith, PhD, University of Florida – \$300,000

- Neuroprotection – boost photoreceptor metabolism
- Gene therapy to provide sustained production of arrestin1

Retinal Progenitors (Neuroprotective for RP)

- jCyte – stem cells that have partially developed into photoreceptors
- Licensing Agreement with Santen
- Injected into the vitreous – release several growth factors
- Rescues cones
- 28 patients treated – moved into Phase 2b (85 participants)



Retinal Progenitors (Photoreceptor Replacement for RP)

ReNeuron (Mass Eye and Ear)

- Transplant partially developed photoreceptors
- Functionally replace lost photoreceptors
- Improved visual acuity for patients in Phase 2 – ~3 lines on an eye chart at 12 months
- Jason Comander leading trial
- Significant funding from FFB

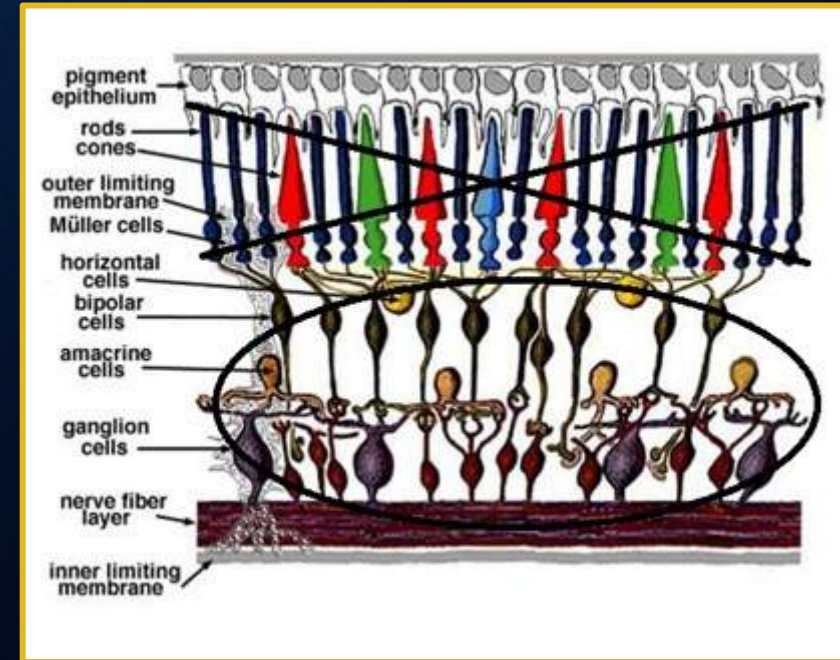
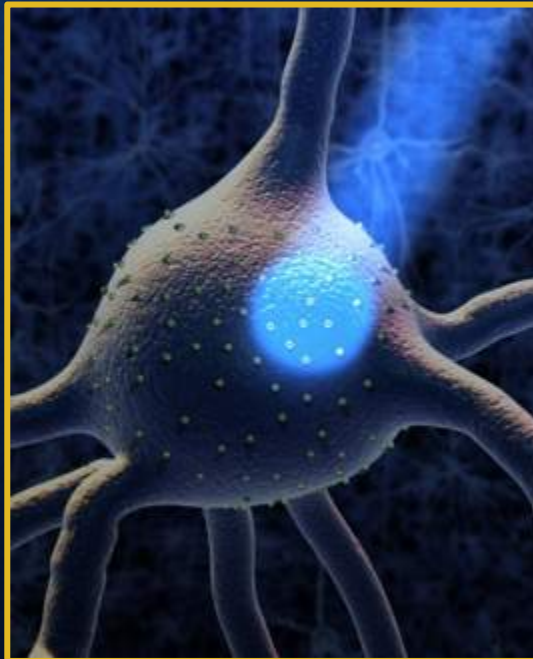


Optogenetic Therapies

Restores light sensitivity to retina affected by advanced disease.

Three Clinical Trials:

- Allergan (US)
- GenSight (UK)
- Bionic Sight
w/AGTC (US)



Resources

Foundation Fighting Blindness

[FightingBlindness.org](https://www.fightingblindness.org)

[ClinicalTrials.gov](https://www.clinicaltrials.gov)

[MyRetinaTracker.org](https://www.myretinatracker.org)