Emerging cross-cutting therapies for Usher syndrome

July 11, 2020

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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
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
ClinicalTrials.gov
MyRetinaTracker.org