



Staying Alive

Saving the Retina through Neuroprotection

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**FOUNDATION
FIGHTING
BLINDNESS**

Can disco save your vision?

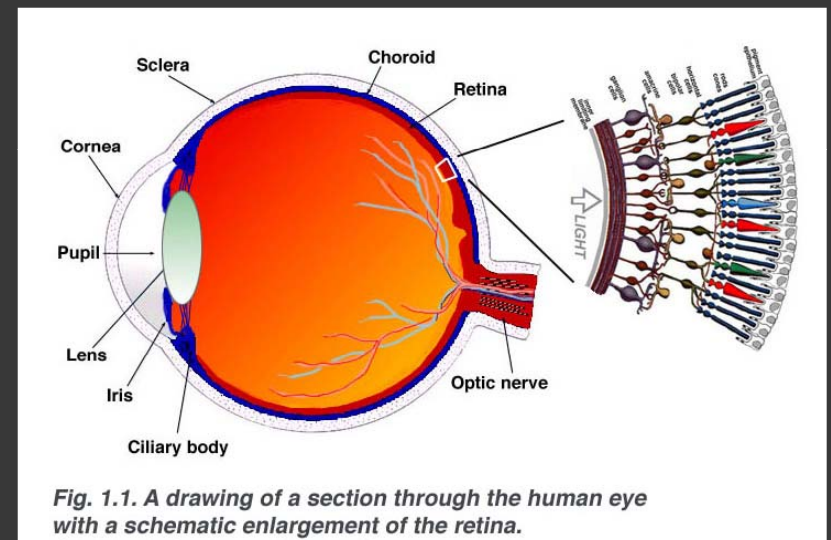
The Foundation Fighting Blindness

- FFB: world's largest non-governmental source of research funding for **all retinal degenerative diseases**
- Funding innovative, cutting-edge research – sight-saving potential
- 114 projects at 69 institutions:
 - Johns Hopkins University (Wilmer Eye Institute)
 - Harvard Medical School (Berman-Gund Laboratory)
 - University College London (Moorfields Eye Hospital)
 - Emory University
 - Medical University of South Carolina

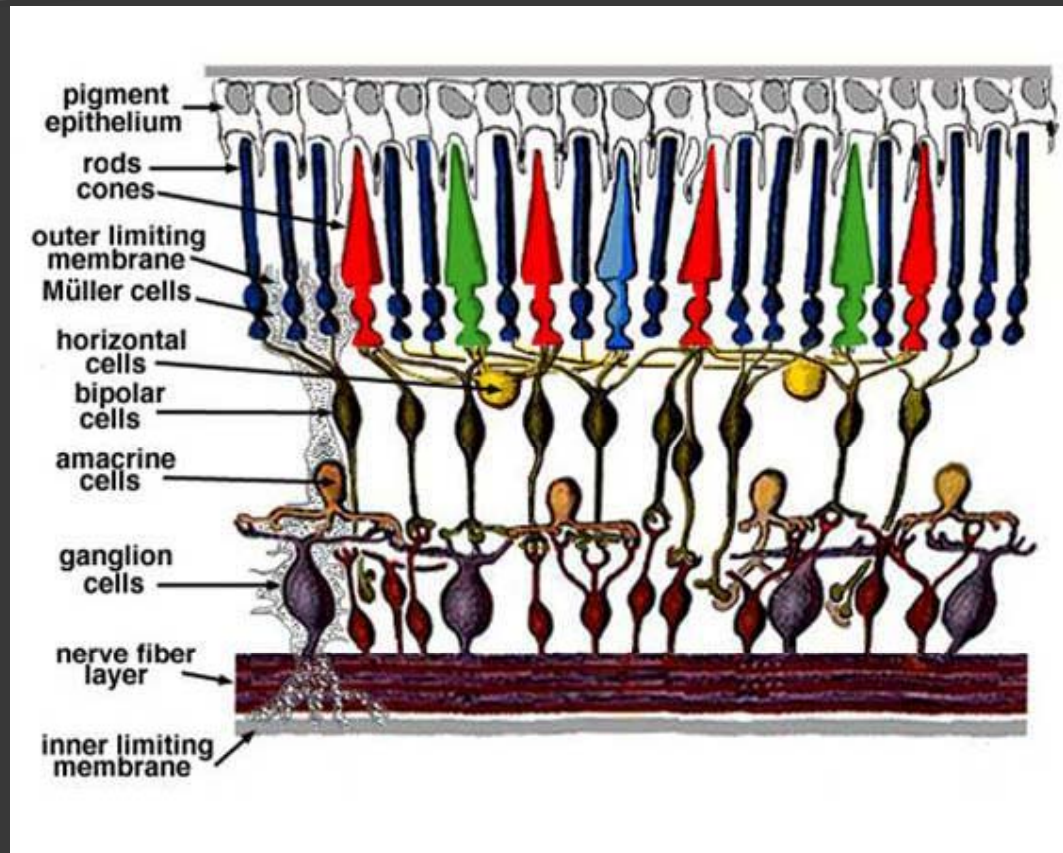
Usher syndrome =
retinitis pigmentosa + hearing loss

The Retina

- Thin, delicate layer of tissue at the back of the eye — only $\frac{1}{2}$ millimeter thick
- Converts images into electrical signals, sending them back to the brain
- Enables us to see — like digital sensors (film) in a camera



The Retina



What is Neuroprotection?

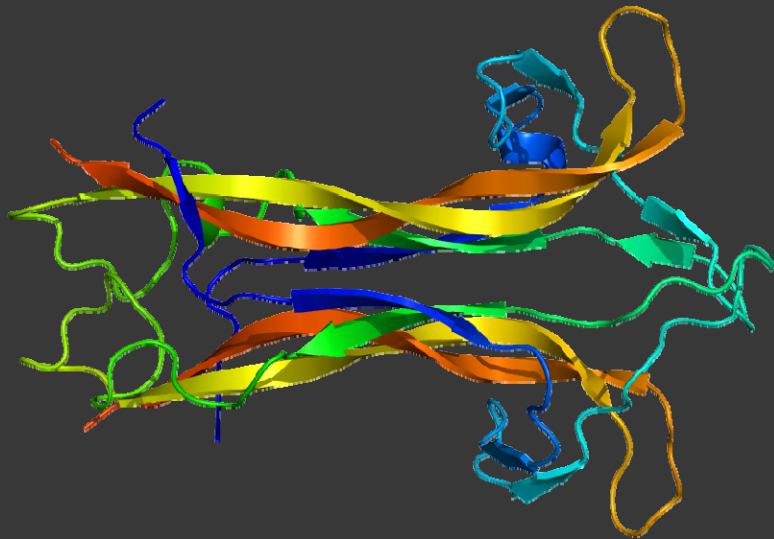
- Delivering small molecules or proteins to keep retinal cells healthy and functional
- Under development for a wide variety of neurodegenerative conditions:
 - Parkinson's disease
 - Alzheimer's disease
 - Multiple sclerosis
 - Glaucoma

Retina is an extension of the brain



What is Neuroprotection?

- Neuroprotective approaches:
 - **anti-oxidants** -- reducing oxidative stress
 - **anti-apoptotics** -- preventing programmed cell death
 - **growth factors** -- promoting growth and healing
 - **boosting mitochondrial function** -- increasing energy supply



BDNF – Brain-Derived Neurotrophic Factor

Neuroprotection Delivery



Eye Drops



Oral Therapy



Cells (Stem Cells)



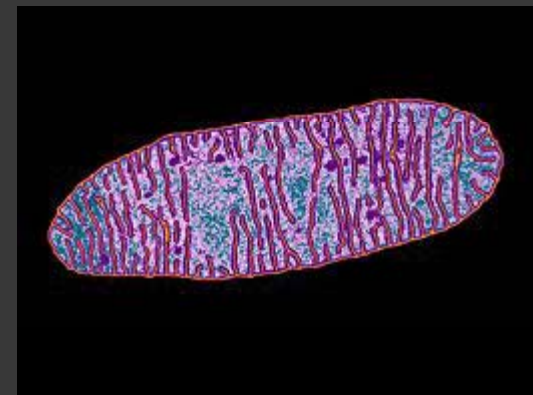
Gene Therapy

Why Neuroprotection?

- Gene replacement, photoreceptor replacement may not be right for patient – not every treatment will be right for everybody, because:
 - Condition of their retinas
 - Clinical issue (e.g., immunological disease)
 - Diagnostic challenge (can't find mutated gene)
- Combination therapy – with gene therapy or stem cells
- Oral or eye drops can be modulated – dosing can be adjusted

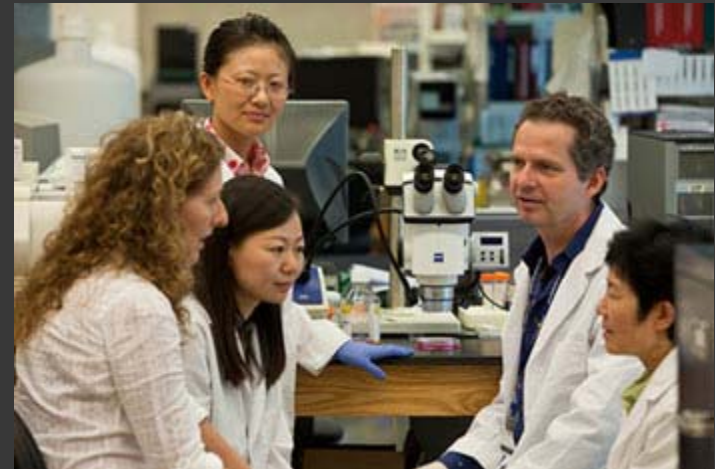
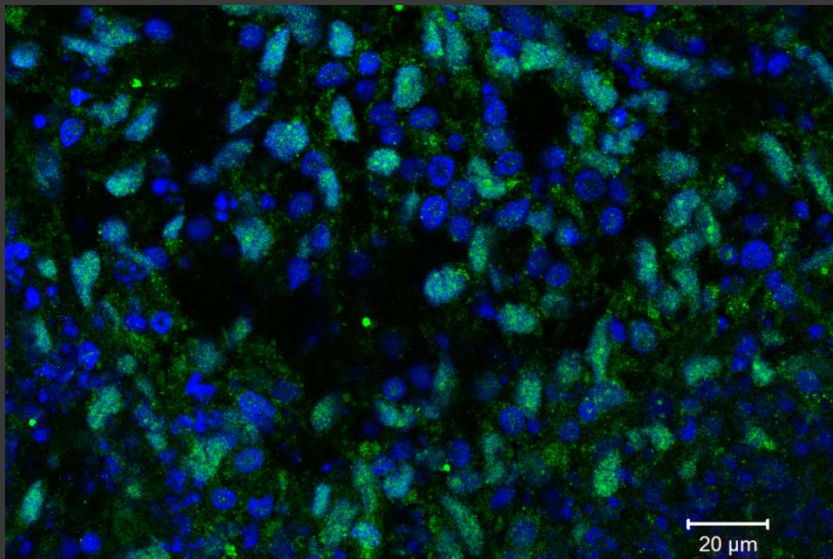
MitoChem Therapeutics

- New biotech spun out from the Medical University of South Carolina – Craig Beeson, Ph.D., Barb Rohrer, Ph.D.
- Developing a small molecule – eye drop that boosts mitochondrial function (organelle produces energy)
- Screened library of 50,000 compounds
- Identified lead compounds – narrowed down to one (CB11)
- Excellent efficacy *in vitro* and *in vivo*
- RP models – large animal (pig)
- Goal: IND to launch clinical trial

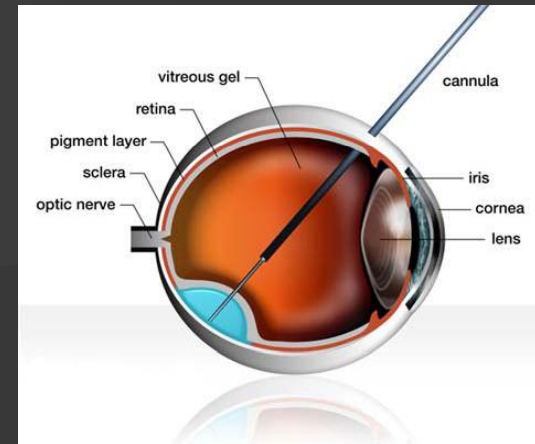


Retinal Progenitors

- Stem cells that have partially developed into retinal cells
- Henry Klassen, Ph.D., University of California, Irvine
- Injected into the vitreous – release several growth factors
- Rescues **cones**
- FDA has authorized clinical trial



Rod-Derived Cone Viability Factor (RdCVF)



- Jose Sahel & Thierry Laveillard, Institut de la Vision in Paris
- Naturally occurring protein that keeps cones healthy
- Gene therapy, but works independent of retinal disease
- AAV: manmade virus that delivers copies of the therapeutic genes – one injection can last several years
- Works like a drug factory in the retina – sustained, continual release

Other FFB-Funded Neuroprotection Projects

- **Plant Extracts:** Thierry Léveillard, Ph.D., Institut de la Vision
 - Targeting cones, identifying specific (most potent) molecule
- **Proteins:** John Ash, Ph.D., University of Florida
 - Gene therapy delivering STAT3 and PIM-1 (antioxidative proteins)
- **Testing Platforms:** Matthew LaVail, Ph.D., UCSF
 - Evaluates collaborator's molecules in animal models

Can Disco Save Your Vision?

YES
(sort of)



Aerobic Exercise Preserves
Vision in Retinal
Degeneration Lab Study

- Pardue, Boatright, et al

For More Information

- www.fightblindness.org (FFB)
- bshaberman@blindness.org (Ben)
- FFB: 1-800-683-5555
- www.clinicaltrials.gov