

National Eye Institute (NEI)

Director: **Paul Sieving, M.D., Ph.D.**
 Deputy Director: **Belinda Seto, Ph.D.**

Mission of the National Eye Institute

To conduct and support research, training, health information dissemination, and other programs with respect to blinding eye diseases, visual disorders, mechanisms of visual function, preservation of sight, and the special health problems and requirements of the blind. Established by U.S. Congress, 1968

Funding

- NEI is the largest vision research organization in the world.
- **\$676 million** = 2014 NEI budget
- **1,382** Grants awarded by NEI to **1,198** principal investigators at **257** universities and research organizations
- **\$580 million** = Funding provided to labs at universities and other research organizations across the country
- **56** Small business grants to research and develop vision-related devices and treatments
- **44** New investigators funded in FY 2014

Blindness and low vision are devastating.

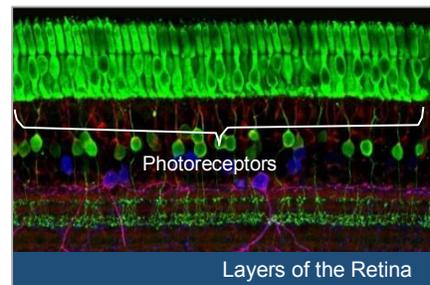
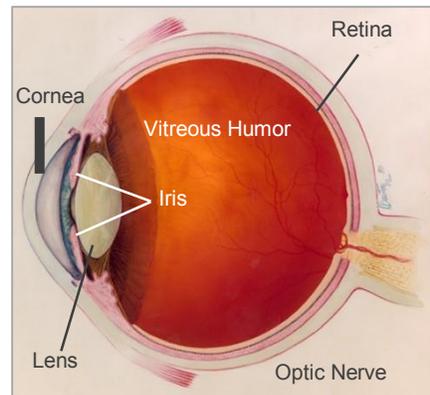
- **1.3 million** Americans are blind ($\leq 20/200$)
- **2.9 million** Americans have low vision ($< 20/40$)
- **\$145 billion** = Estimated annual U.S. economic burden

The National Eye Health Education Program stresses early detection.

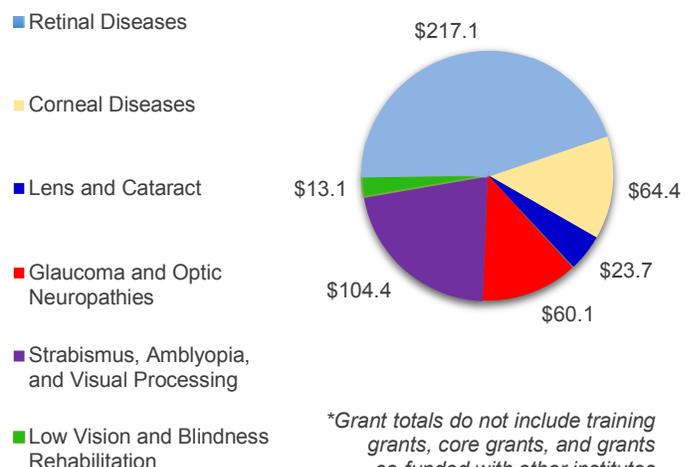
Created in response to a 1988 directive from Congress "to increase [NEI's] commitment to the prevention of blindness through public and professional education programs and the encouragement of regular eye examinations."

NEI Audacious Goals Initiative will set priorities.

- **The goal: To Regenerate Neurons and Neural Connections in the Eye and Visual System**
- Catalyzing vision research to develop treatments that restore vision to patients with blinding diseases in the next 10-15 years.
- AGI targets: 1) Photoreceptors; 2) Retinal Ganglion Cells and Optic nerve.
- AGI Steering Committee expertise includes stem cells, medicine, neurodevelopment and circuitry.
- Scientific workshops guide funding opportunities.
- First round of collaborative grants will develop new real-time imaging tools to test therapies.
- Growing and connecting functional nerve cells is central to all neuroscience.



FY14 Grants* (\$ millions)





Vision Diseases and Research Progress

Vision Research Leads the Way

- **Genetics:** Discovery of more than 500 genes related to eye diseases is unparalleled in disease research. NEI grantees pioneered genomics studies for complex diseases.
- **Gene Therapy:** First successful gene therapy clinical trial for Leber congenital amaurosis, a form of childhood blindness, with other disease trials underway.
- **Cell Therapy:** Patients' cells converted to retinal neurons for therapeutics and research.
- **Neuroscience:** Retina is part of the brain that we can see directly; it holds the key to understanding brain circuits.

Age-related Macular Degeneration (AMD)

Leading cause of blindness in older Americans.

- First successful genome-wide association study for a complex disease identified major genetic risk factor, especially in smokers, opening the door to potential AMD therapies.
- Comparative effectiveness trial found cancer drug Avastin is as effective as Lucentis for improving vision in advanced AMD.
- Age-Related Eye Disease Study showed that daily antioxidant and mineral supplementation delays severe vision loss in AMD by 25 percent over five years.
- Repurposing FDA-approved HIV drugs show promise for treating AMD.

Glaucoma

Optic nerve degenerates, often due to high fluid pressure in the eye.

- Two new genetics studies seek to understand why African Americans and Hispanics have a 4- to 10-fold greater incidence and greater risk of blindness.
- Largest genomic study of glaucoma shed light on complex genetic inheritance.
- Clinical trials confirmed efficacy of pressure-reducing drugs for glaucoma.

Diabetic Retinopathy

Complication of diabetes is leading cause of blindness in working-age adults.

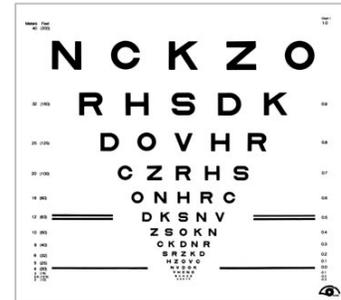
- Diabetic Retinopathy Clinical Research Network demonstrated that Lucentis plus standard laser therapy can improve vision, providing the first new therapy in 20 years.
- Recent trial compared effectiveness of Lucentis, Eylea, and Avastin for diabetic macular edema. All three were equally effective for mild vision loss; Eylea was superior for severe vision loss.
- Collaboration with the National Heart, Lung, and Blood Institute found that tighter control of blood sugar and two lipid-lowering therapies significantly reduces vision loss.

Pediatric Eye Disease

- Pediatric Eye Disease Investigator Group trial for amblyopia—a disorder where the brain favors one eye over the other—found atropine eye drops are as effective as wearing an eye patch, improving treatment compliance in children.
- Better diagnostic tests and telemedicine approaches for monitoring and delivering timely treatment to premature infants at risk for retinopathy of prematurity.

NEI is Poised for Precision Medicine

- EyeGENE Network, diagnostic DNA sequencing for patients with rare eye diseases.
- Large patient cohorts for genomics in complex diseases such as glaucoma and AMD.
- Proof-of-concept and pioneering research for stem cell therapies and gene therapies.
- New research programs in Molecular Therapy for Eye Diseases; Aging and Eye Biology.



Normal Vision



Age-related Macular Degeneration



Glaucoma



Diabetic Retinopathy



Cataract