

# Foundation Fighting Blindness, Inc.



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## Mission

The mission of the Foundation Fighting Blindness (FFB) is to drive the research that will provide preventions, treatments, and cures for people affected by retinitis pigmentosa (RP), macular degeneration, Usher syndrome, and the entire spectrum of retinal degenerative diseases. Historically, the Foundation has played a unique role in advancing the most exciting new research programs to a stage where they will attract external support from industry or government in order to reach the clinic.

## Strategy/Approach

In order to achieve its mission, the Foundation funds pioneering research in six priority areas:

- Gene Therapy
- Regenerative Medicine
- Novel Medical Therapies
- Clinical: Structural and Functional Relationships
- Cell and Molecular Mechanisms of Disease
- Genetics

In addition to the research priority areas, the Foundation supports several “cross-cutting” efforts, including data and research sharing, interdisciplinary research approaches, and career training programs. FFB ultimately seeks to fund cutting-edge research; develop new treatments and cures; identify new therapies; improve diagnoses; foster education, awareness, and support; and attract the public to its mission.

FFB also created the Foundation Fighting Blindness Clinical Research Institute, a nonprofit support subsidiary, to accelerate the translation of fundamental research into clinical trials for inherited retinal degenerative diseases and, ultimately, to accelerate the availability of patient therapies. The institute fosters collaborations among the scientific, clinical, governmental, pharmaceutical, financial communities and provides support and expertise in validation of new clinical endpoints, in clinical development strategies and regulatory strategies. As part of this effort, the Foundation has established an on-line interactive patient registry, My Retina Tracker, which provides resources for patients, researchers, and clinicians.

The Foundation has set up milestones for its activities under a 2011-2016 Strategic Plan. By 2016, FFB aims to have played a major role in adding at least 10 new clinical trials to its translational research portfolio, in addition to seven current trials.

## Research Portfolio

FFB supports basic, translational, and applied research that advances therapeutic and preventive strategies into the clinic.

The 2014 research program includes:

- **Foundation Center Grants** – Foster the collaborative efforts of independent research institutions by pairing basic scientists with clinical investigators and enabling them to better share knowledge and resources to more effectively develop promising treatments and cures.
- **Alan Lattes Career Development Program** – Supports the development of clinician-scientists at all stages of their career, from medical student to tenured faculty, conducting research in inherited retinal disorders by supporting talented and ambitious young clinicians during the years leading up to an independent research program.
- **Individual Investigator and Collaborator Awards** – Foster the advancement of therapeutic and preventive strategies into the clinic by supporting basic, translational, and applied research in inherited orphan retinal degenerative diseases and dry age-related macular degeneration.
- **Research Facility Awards** – Foster the concentration of resources to drive the research to prevent, treat, and cure inherited retinal degenerations and macular degenerations.
- **Early Translational Research Program** – Accelerates the development and pathway into clinical trials for treatments for inherited retinal diseases.
- **Foundation Fighting Blindness Clinical Research Institute Grants** – Awarded through the Foundation Fighting Blindness Clinical Research Institute to support the pre-clinical and human proof of concept studies to drive the translation of promising scientific accomplishments from bench to bedside.

Other research activities include:

- **The National Eye Evaluation Research Clinical Trial Network** – Established under the Foundation Fighting Blindness Clinical Research Institute to advance the science of therapeutic and preventive interventions through the conduct of clinical trials and other clinically relevant studies.
- **RetNet** – The retinal information network providing public access to information on the genes causing inherited retinal diseases, such as retinitis pigmentosa, macular degeneration, and Usher syndrome.
- **Genetic Testing Services** – Supports multiple options for genetic testing for inherited orphan retinal degenerative diseases.

## Partnership Practices

The Foundation engages in corporate, academic, and nonprofit alliances. **The Foundation has a number of successful collaborations with biotech and pharmaceutical companies.** It supplies “Valley of Death” investment and preclinical and clinical infrastructure to ensure no promising treatment is overlooked in developmental programs. Examples include:

- **MitoChem Therapeutics** – A \$2 million investment in the Charleston, S.C.-based start-up company to develop a cross-cutting treatment for vision-robbing retinal degenerative diseases.
- **Applied Genetic Technologies Corporation** – A \$1.5 million grant to this clinical-stage biotechnology company supported a preclinical trial evaluating the safety and efficacy of a gene therapy treatment for X-linked Retinoschisis.
- **Oxford BioMedica** – Funded early-stage preclinical program for StarGen™, a gene therapy for Stargardt disease, and for UshStat, a gene therapy for Usher syndrome, both now in clinical trials, also supported by the Foundation.
- **Genable Technologies** – Funding early-stage preclinical program of gene therapy for autosomal dominant retinitis pigmentosa.
- **ReNeuron Group**—Supporting the development of ReN003, a retinal stem cell therapy candidate for retinitis pigmentosa, to undergo clinical trials.
- **Howard Hughes Institute-Foundation Fighting Blindness Medical Fellowship Award**—Funds up to four research fellowships for medical students through the HHMI Research Training Fellowships program to foster the development of physician scientists in this area.

## Financials

The Foundation is a 501(c)(3) nonprofit health organization. The most recent financial information available is from 2013.<sup>1</sup>

Year ending 06/30/13:

- Revenue: \$44,754,000
- Assets: \$75,158,000
- Research: \$17,502,000

## Key Accomplishments

- Funds thousands of research studies at hundreds of prominent institutions worldwide. Currently, the Foundation funds 122 grants at 69 institutions across seven countries.
- Funded the research that has led to the discovery of more than 80 percent of the genes currently known to be affected in the inherited orphan retinal degenerative diseases.
- Has raised more than \$550 million as the leading non-governmental funder of retinal research since its founding in 1971.

- Provided critical funding into and through groundbreaking clinical gene therapy trials for Leber’s congenital amaurosis that restored vision to children and young adults who were virtually blind.
- Provided critical early translational research funding that led to the first gene therapy trial for choroideremia in the UK and supported the late stage development of the choroideremia gene therapy trial about to start in the US.
- Through targeted funding, primed the StarGen™ program, at Oxford BioMedica – leading to a \$50 million follow-on investment by Sanofi.
- 50 volunteer-led chapters across the United States that raise funds, increase public awareness, and provide support to its communities.
- 2013 marked the second year in a row that the Foundation invested more than \$20 million into research and public health

## Leadership

The Foundation is governed by a Board of Directors, counseled by a Scientific Advisory Board, and managed by a Maryland-based national leadership staff. It also maintains eight regional offices around the country.

- **Chief Executive Officer:** William Schmidt, [bschmidt@fightblindness.org](mailto:bschmidt@fightblindness.org)
- **Chief Development Officer:** James Minow, [jminow@fightblindness.org](mailto:jminow@fightblindness.org)
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- **Chief Financial Officer:** Annette Hinkle, [ahinkle@fightblindness.org](mailto:ahinkle@fightblindness.org)

<sup>1</sup> Information obtained from 2013 Annual Report. <http://www.blindness.org/2013AnnualReport/>, March 2014.