Victories for Vision

2021 ANNUAL REPORT

50 YEARS OF FIGHTING

FOUNDATION FIGHTING BLINDNESS

Together, we're winning.

For 50 years, we've fought to find preventions, treatments and cures for people affected by retinitis pigmentosa, macular degeneration, Usher syndrome and the entire spectrum of blinding retinal diseases. In that time, the science has led to brilliant breakthroughs and established many research pathways to pursue.

We've come so far—but there's so much left to do. These important breakthroughs would stop without research funded by the Foundation Fighting Blindness... with the research made possible by you.

Learn more by calling (800) 683-5555 or visiting FightingBlindness.org to sign up for the latest updates.

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A Message from Our Chief Executive Officer

Looking back over this past year, I cannot help but ponder the 50 years that brought us to this point.

For any organization, nonprofit or not, to be viably in business for half a century is remarkable. The fact that we have been able to fund and engage in a specific, narrow space for all this time is a testament to our board, staff, volunteers, and donors. It is not unusual for me to meet people who have been involved for 40 or 50 years.

I'm convinced that it's this kind of long-term engagement and support that has helped our organization persevere. The fiscal year that ended June 30, 2021, has shown me that our determination is stronger than ever — reflected in our continued ability to raise funds and by the growing number of clinical and pre-clinical programs in development by industry.

Topping the list of science and research highlights from the past year is the exciting work around optogenetics — the science of restoring vision to completely blind eyes where there are no photoreceptors left. Patients who have been following us for a long time now may have some opportunity for therapy thanks to the work we have funded in this area.

People think of us as an academic funder, but if you only fund academic programs, you don't have clinical programs for patients. So, we are really excited about the number of programs coming from academics to industry. Our investment in ProQR, which is developing RNA therapies to treat inherited retinal diseases, including Leber congenital amaurosis and Usher syndrome, is equally exciting. That's not to mention the gene therapy products being developed by Vedere Bio, which received significant investment from our venture arm, the RD Fund (Retinal Degeneration Fund) and was the first successful exit of the first 10 companies we funded.

If all that wasn't enough, in April, we hired Claire M. Gelfman, PhD, as our new chief scientific officer. She is a career vision scientist with experience in both academia and industry, working on translational drug development in ophthalmology. Just as important, she understands how to translate discovery into a product.

As you will read in these pages, the pace of the science is breathtaking, and the breakthroughs are thrilling — **but it's all made possible by people like you.** Your dedication and support are what spur us on and puts more fire in our belly. I am honored to present you with the Foundation Fighting Blindness 2021 Annual Report.

Sincerely,

Benjamin R. Yerxa, PhD
Chief Executive Officer
Foundation Fighting Blindness
RD Fund

A Message from Our Board Chair

As board chair, it is my honor to introduce the Foundation's 2021 Annual Report. In it, you'll find a summary of the amazing achievements made possible over the last year by the research we fund.

Our success to this point is the cumulative result of five decades of hard work and groundbreaking research. Reaching this 50th anniversary is all the more exciting because of the victories we're starting to see — made possible by a combination of your ongoing support and stellar leadership.

CEO Ben Yerxa has helped us build a modern-day, entrepreneurial nonprofit that is collegial and inclusive. As a result, we can now execute our strategic vision like never before. Now we're expanding our investments, supporting more young researchers, and nurturing new ideas on the bench.

Since the Foundation has gotten involved in gene therapy, millions of dollars have been invested in the work. And it's recently been announced that our own RD Fund has launched Opus Genetics, a new company that will focus on treatments for pediatric blinding diseases. Our role is to expand the field and raise more funds to move it even further forward.

In this 50th year, it's as important as ever to acknowledge the remarkable efforts of the board of directors. I've never seen a board so committed to the field for such a long time. Supporters should be proud of the board leadership.

This is a journey that requires all of us. Which is why, in addition to my role on the board, I have agreed to serve as a co-chair of the Victory for Vision campaign. In conjunction with the Foundation's 50th anniversary, the campaign seeks to raise an additional $50 million above our annual fundraising — funds that will help us keep pace with the rapidly advancing science we've been nurturing since 1971.

As a friend of the Foundation, you know how far we've come, but also how much we have left to go. I'm here to encourage all of us to keep our focus on the future.

Twenty years ago, I would have told you I had no idea if we could do this. But today, it isn't unreasonable to think that in my lifetime, we will have found treatments or cures for most retinal diseases.

Thanks to supporters like you, I am hopeful. Together, we will have victory for vision.

With gratitude,

David Brint
Chair, *Board of Directors*
Co-Chair, *Victory for Vision Campaign*

Research Progress for 2021

While the COVID-19 pandemic presented challenges and delays for all areas of biomedical research, clinical development for retinal degenerative disease treatments over the past year continued to move forward impressively. Foundation funding continues to play a leading role in advancing the field, especially in moving emerging therapies into and through clinical trials. More than 43 clinical trials are underway for potential treatments for inherited retinal diseases (including dry AMD treatments).

This fiscal year's research highlights include:

Encouraging Early Report for Three Patients in Atsena's LCA1-GUCY2D Gene Therapy Clinical Trial

A clinical research team led by Samuel Jacobson, MD, PhD, at the University of Pennsylvania, observed vision improvements for the first three patients with Leber congenital amaurosis 1 (LCA1) treated with an emerging GUCY2D gene therapy in a Phase 1/2 clinical trial. The gene therapy is being developed by Atsena Therapeutics, a clinical-stage gene therapy company. The therapy was created in the laboratory of Atsena Founder and Chief Scientific Officer Shannon Boye, PhD, and Founder and Chief Technology Officer Sanford Boye, M.Sc., at the University of Florida. The Foundation Fighting Blindness RD Fund is an investor in the company.

Stargazer Pharmaceuticals Initiates a Phase 2a Clinical Trial for its Stargardt Disease Drug

Stargazer Pharmaceuticals, Inc., a Boston-based biopharmaceutical company developing treatments for rare eye diseases, initiated a Phase 2a clinical trial of STG-001, an indirect visual cycle modulator, in Stargardt disease patients. The clinical study is evaluating two doses of STG-001 with regard to safety, the drug's biodistribution in the body, and its mechanism of action. The Foundation Fighting Blindness RD Fund is an investor in the company.

ProQR's RNA Therapy for USH2A Performs Well in Phase 1/2 Clinical Trial

ProQR, a developer of RNA therapies in the Netherlands, has reported improvements in vision and retinal structure for patients in its Phase 1/2 Stellar clinical trial for QR-421a, an RNA therapy for people with mutations in exon 13 of the USH2A gene. These mutations cause Usher syndrome type 2A (USH2A) or non-syndromic retinitis pigmentosa (RP). According to ProQR, more than 16,000 in the Western world have USH2A or RP caused by these mutations. As a result of these findings, ProQR is planning two Phase 2/3 trials for QR-421a. The Foundation Fighting Blindness Retinal Degeneration Fund invested $7.5 million to move QR-421a into and through the early stage clinical trial.

Bionic Sight's Optogenetic Therapy Enables Blind Patients to Detect Light and Motion in Early Trial

Four patients with retinitis pigmentosa (RP) – all of whom had complete or nearly complete blindness – had some vision restored in a Phase 1/2 clinical trial for an emerging optogenetic therapy developed by Bionic Sight. All of the patients can now see light and motion. Two of the patients can detect the direction of motion; that is, they can determine if objects are moving to the right or left. The company's trial is the first to report vision restoration in humans receiving an optogenetic therapy. The therapy is designed to work for people with advanced vision loss, independent of the mutated gene causing the retinal disease.

Investigators Report Partial Vision Restoration for Patient in GenSight Biologics' Optogenetic Therapy Trial

Clinical researchers testing an experimental therapy developed by GenSight Biologics, a clinical-stage gene therapy development company, have reported that one patient with retinitis pigmentosa (RP) demonstrated partial vision restoration in the Phase 1/2 PIONEER clinical trial for its emerging optogenetic therapy. Results from the study were reported in the journal *Nature Medicine*. The Foundation funded preclinical research that led to the initiation of this important clinical trial.

REGENXBIO's Wet AMD Gene Therapy Moves into Phase 3 Trial

REGENXBIO, a clinical-stage gene therapy biotechnology company, launched its first Phase 3 clinical trial for RGX-314, its gene therapy designed to halt the growth of leaky blood vessels that cause retinal degeneration and central vision loss in people with the wet form of age-related macular degeneration (AMD). REGENXBIO's emerging gene therapy is designed to greatly reduce the need for repetitive anti-VEGF injections. RGX-314 is administered as a one-time subretinal injection in the affected eye and provides sustained production of the anti-VEGF protein.

EYE ON THE CURE

Eye on the Cure Podcast

The new Eye on the Cure Podcast from the Foundation Fighting Blindness provides science information, news, and insights from the world of vision and retinal diseases. The podcasts are hosted by Ben Shaberman, senior director scientific outreach, and often include conversations with guests from the research and vision communities. Stream the Foundation Podcasts on SoundCloud, Spotify, Audible, Pandora, and more: FightingBlindness.org/Podcasts

Scan the QR code to go directly to the web page on your device.

An Eye on Education Video Series

Learn all about inherited retinal diseases and related science topics by watching (or listening to) the Foundation's educational video series, An Eye on Education, developed for patients, families, and other lay audiences. Watch these videos by visiting: FightingBlindness.org/EducationalVideos

Scan the QR code to go directly to the web page on your device.

Clinical-Trial Pipeline

Inherited Retinal Diseases and Dry AMD: 43 Trials (select)

Below includes many of the clinical trials of emerging therapies underway for inherited retinal diseases and dry age-related macular degeneration. For more details on these trials, visit: ClinicalTrials.gov and FightingBlindness.org.

GENE THERAPIES (GENE TARGET)	PROGRESS
Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – AGTC	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD-dry – Gyroscope	Phase 2
Choroideremia (REP1) – 4DMT	Phase 1/2
Choroideremia (REP1) – Spark	Phase 1/2
Choroideremia (REP1) – Tubingen Hosp	Phase 2
LCA (CEP290, CRISPR) – Editas	Phase 1/2
LCA (GUCY2D) – Atsena	Phase 1/2
LCA and RP (RPE65) – MeiraGTx/Janssen	Phase 1/2
RP (PDE6B) – Coave	Phase 1/2
RP (RLBP1) – Novartis	Phase 1/2
RP (PDE6A) – Tubingen Hosp	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 1/2
X-linked RP (RPGR) – MeiraGTx/Janssen	Phase 1/2
X-linked RP (RPGR) – 4DMT	Phase 1/2

RNA/OTHER THERAPIES (MECHANISM)	PROGRESS
AMD-dry (CB inhibitor) – Ionis	Phase 2
AMD-dry (C5 inhibitor) – Iveric bio	Phase 2
LCA (CEP290, RNA) – ProQR	Phase 2/3
RP (RHO, RNA) – ProQR	Phase 1/2
RP, Usher, others (optogenetic) – Allergan	Phase 1/2
RP, Usher, others (optogenetic) – Bionic Sight	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP, Usher, others (optogenetic) – Nanoscope	Phase 2
Usher syndrome 2A (RNA) – ProQR	Phase 1/2

CELL-BASED THERAPIES (CELL TYPE)	PROGRESS
AMD-dry (RPE) – Astellas	Phase 1/2
AMD-dry (RPE) – Cell Cure	Phase 1/2
AMD-dry (RPE from iPSC) – NEI	Phase 1/2
AMD-dry (RPE on scaffold) – Regen Patch	Phase 1/2
RP, Usher (retinal progenitors) – jCyte	Phase 2b
RP, Usher (retinal progenitors) – ReNeuron	Phase 2
Stargardt (RPE) – Astellas	Phase 1/2

SMALL MOLECULES (MECHANISM)	PROGRESS
AMD-dry (C3 inhibitor) – Apellis	Phase 3
RP (NAC-anti-oxidant) – Johns Hopkins	Phase 2
Stargardt disease (emixustat) – Acucela	Phase 3
Stargardt disease (deuterated vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Iveric bio	Phase 2
Stargardt disease (anti-RBP4) – Belite Bio	Phase 1
Stargardt disease (anti-RBP4) – Stargazer	Phase 2
Stargardt disease (metformin) – NEI	Phase 1/2
Usher syndrome (NACA-anti-oxidant) – Nacuity.	Phase 1/2

A Message from Our Chief Scientific Officer

When I joined the Foundation in April as chief scientific officer, I knew I had tremendous shoes to fill. The Foundation's 50-year legacy of driving sight-saving and sight-restoring research is unparalleled. No other organization has come close to doing more to advance treatments and cures for inherited retinal diseases. I am incredibly honored to join the amazing team at the Foundation who work tirelessly towards our urgent mission.

My career in ophthalmology has spanned academia, contract research organizations, and the pharmaceutical industry, with the common thread of developing the most innovative treatments for blinding diseases. I look forward to leveraging my experience in the retinal gene therapy industry – I was most recently vice president of pharmaceutical development at Adverum Biotechnologies, an ocular gene therapy company – to help guide the Foundation's research efforts to get more therapies into the pipeline, across the finish line, and out to the people who need them.

In addition to making my contributions, I look forward to learning from the incredibly broad and deep experience of the Foundation's staff, Scientific Advisory Board, Research Oversight Committee, and global network of retinal researchers. I am truly fortunate to have such a dedicated team of experts to work with every day. My immediate goals have been to familiarize myself with the Foundation's vast research portfolio, connect with the dozens of researchers in our space, and meet the patients and families that so passionately drive our mission.

From day one, my main goal has been to help accelerate research into and through clinical trials by funding the best science with the most potential to save and restore vision. That is my focus with every decision we make and every dollar we spend. I am excited that we can take advantage of the many promising therapeutic modalities in development – including gene therapies and gene editing, cell-based therapies, and pharmaceuticals – to ultimately help everyone diagnosed with inherited retinal diseases and dry age-related macular degeneration, regardless of their gene mutation and degree of vision loss.

I am also deeply committed to helping our constituents understand the science and the impact of their donations.

While there is tremendous progress with so many clinical trials underway, a lot of work remains ahead. It is my privilege to do all I can to get more therapies to those who need them.

I am delighted to be a member of the Foundation's family and hope to meet many of you soon.

Thank you,

Claire M. Gelfman, PhD
Chief Scientific Officer

Foundation Awarded 20 New Grants in FY2021 Totaling More than $13.9 Million

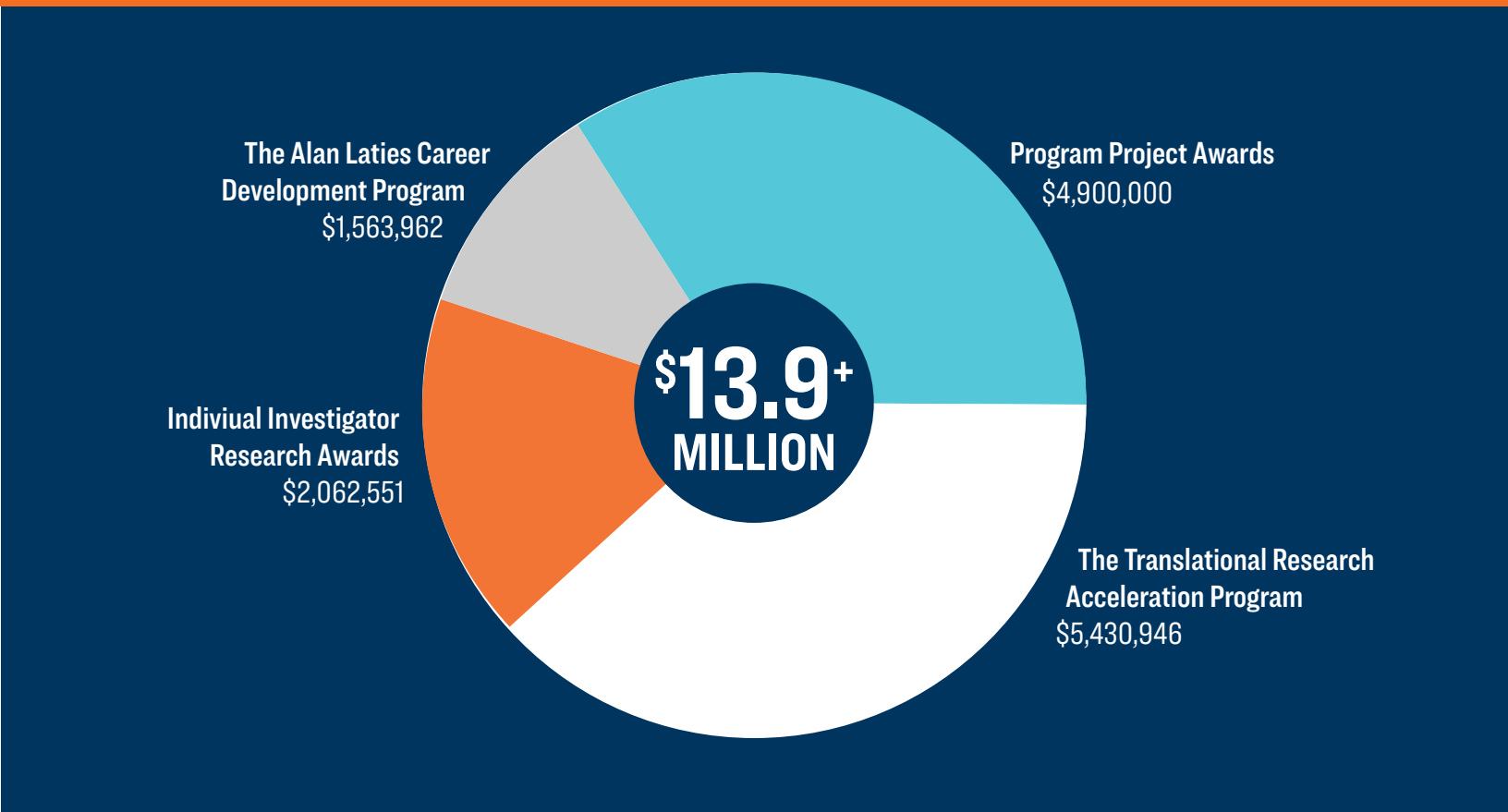
During its fiscal year 2021, the Foundation approved 20 new research projects to its portfolio, with awards ranging from early stage lab research efforts to translational studies for advancing emerging therapies toward clinical trials.

The Foundation funds a diverse portfolio of emerging therapies to address the entire spectrum of inherited retinal diseases and dry AMD for all patients affected regardless of the mutated gene causing their disease or the severity of their vision loss.

Though the overarching goal of the Foundation is to develop treatments and cures, we also fund projects to: 1) better understand disease mechanisms and therapeutic targets, 2) develop models for testing treatments, and 3) evaluate disease progression to identify patients and outcome measures for clinical trials.

Research grants are selected after a rigorous review process conducted by the Foundation's Scientific Advisory Board, which is comprised of more than 50 of the world's leading retinal scientists and clinicians.

New Awards
Fiscal Year 2021



Types of Awards

The Alan Laties Career Development Program supports physicians' and physician-scientists' career development through a multi-level, tiered approach across the duration of their career. Each level represents a new "stage" in the career progression of a physician or physician-scientist, at a point when they can choose a clinical and/or research direction. The Foundation Fighting Blindness' career development program includes funding support for clinicians, and medical residents in veterinarian studies in retinal degenerations.

Individual Investigator Research Awards are designed to concentrate research in areas with the greatest potential to move toward treatments and cures for the inherited orphan retinal degenerative diseases and dry age-related macular degeneration.

The Translational Research Acceleration Program (TRAP) awards will accelerate the movement of preclinical research toward an Investigational New Drug filing and into clinical trials to provide a robust and diverse pipeline of potential therapies to fight inherited retinal degenerations and dry age-related macular degeneration.

Program Project Awards fund studies that are too large or technically complex for a single investigator to undertake in a reasonable amount of time and to address current knowledge and/or therapeutic gaps.

New Grants

Career Development Awards

Oleg Alekseev, MD, PhD — $65,000
Duke University
Vitamin A Supplementation to Alleviate Night Vision Impairment in Choroideremia Patients
Dr. Alekseev is investigating the use of vitamin A to improve night and peripheral vision in choroideremia patients. He will also study potential visual function outcome measures for use in choroideremia clinical trials.

Susanne Roosing, PhD — $374,999
Radboud University Medical Center
Exploring Ectopic Gene Expression as a Novel Disease Mechanism in Autosomal Dominant Retinal Disease
Dr. Roosing's project aim is to uncover elusive genes that cause inherited retinal diseases caused by complex structural variants or chromosomal rearrangements that lead to errors in or wrongly expressed genes in the retina.

Maximillian Pfau, PhD — $373,963
Institute of Molecular & Clinical Ophthalmology Basel (IOB)
Development and Validation of Visual Function Outcome Assessments for Inherited Retinal Diseases
Dr. Pfau is developing highly sensitive visual function assessments to document the therapeutic benefits of potential therapies for individuals with inherited retinal diseases. Among his goals, he is establishing and validating artificial intelligence-enhanced fundus-controlled perimetry (microperimetry) testing with a focus on Stargardt disease and Usher syndrome type 2A.

Brian Ballios, MD, PhD — $375,000
University of Toronto
Controlling Differentiation of Photoreceptor Progenitors for Retinal Regeneration
Dr. Ballios is focusing on developing and controlling the process in which stem cells produce rod and cone photoreceptors. He is striving to produce populations of cells that are better at engrafting and restoring function to diseased retinas.

Boris Rosin, MD, PhD — $375,000
University of Pittsburgh
Harnessing the Central Nervous System in the Treatment of Inherited Retinal Dystrophies
Dr. Rosin is studying the changes in visual pathways and visual circuit plasticity of the central nervous system prior to and following the application of gene therapy in animal models of inherited retinal diseases, including a mouse model of retinitis pigmentosa. The approach has the potential to lead to better gene and cell therapies for IRDs by maximizing the potential for vision restoration.

Individual Investigator Research Awards

Frauke Coppieters, PhD — $290,000
Ghent University
Design of a Novel Antisense Oligonucleotide Therapy for Inherited Retinal Diseases
Dr. Coppieters' goal is to design and validate a novel, mutation-independent antisense oligonucleotide (AON) therapy which will target specific parts of the genome that control retinal RNA expression and, ultimately, protein production for potentially treating a broad range of inherited retinal diseases.

Marina Gorbatyuk, PhD — $299,338
University of Alabama Birmingham
Targeting Tribbles Homolog 3 Protein for Slowing Retinal Degeneration
Dr. Gorbatyuk is using a multipronged approach to test the hypothesis that TRIB3 inhibition, or reduction of TRIB3 activity, will be neuroprotective to the degenerating retina, regardless of the underlying genetic cause. Her project will include the evaluation of an FDA-approved drug for reducing TRIB3 activity.

Astra Dinculescu, PhD — $300,000
University of Florida
Generation and Characterization of Swine Models of Usher Syndrome Type 3A
Dr. Dinculescu is developing an animal model that recapitulates aspects of human USH3A, thus allowing for the execution of experiments to not only study the pathobiology of the disease but also to provide an in vivo system for the testing of potential therapeutics.

Sina Farsiu, PhD — $300,000
Duke University
Automated Software for Analysis of Adaptive Optics Scanning Optical Coherence Tomography Images
Dr. Farsiu is developing fully automated computational methods and software to quantify photoreceptors captured in three-dimensional images using adaptive optics scanning optical coherence tomography, a high-resolution imaging modality. He will integrate the final algorithms into a free and easy-to-use software package.

Qin Liu, PhD — $300,000
Mass Eye and Ear
Gene Editing Approaches for the Treatment of Inherited Retinal Diseases
Dr. Liu is developing gene-editing strategies for the treatment of retinitis pigmentosa caused by mutations in RP1, the third most common dominant RP gene. This approach is applicable for other types of autosomal dominant RP as well as other inherited retinal diseases with autosomal dominant inheritance.

Martin Pera, PhD & Patsy Nishina, PhD — $578,213
The Jackson Laboratory
Precision Functional Genomics for Modeling Pathogenesis of Age-Related Macular Degeneration
Dr. Pera and Dr. Patsy Nishina are creating models of age-related macular degeneration to investigate a potential cellular pathway that may be critical for developing AMD and to assess AMD genetic causes. They are using a precision medicine approach to generate multiple strains of mouse stem cell-derived retinal pigment epithelial cells that are deficient in a known genetic risk factor for AMD called TIMP3. Based on this outcome, they will then determine if they can predict the consequences of these mutations in the development of disease.

Translational Research Acceleration Program Awards

Stephen Tsang, MD, PhD — $300,000
Columbia University
Development of a Cross-Cutting Gene Therapy for Retinitis Pigmentosa
Dr. Tsang is developing a gene therapy to increase aerobic glycolysis — a process that generates energy — in cone photoreceptors of those affected by retinitis pigmentosa. He believes the approach may preserve cones for RP patients and would do so independent of the mutated gene causing the disease.

Bärbel Rohrer, PhD — $342,389
Medical University of South Carolina
Development of a Dry AMD Gene Therapy
Dr. Rohrer is conducting an animal study of a gene therapy designed to selectively deliver a component of complement factor H (CFH) to temper the overactive innate immune system in age-related macular degeneration. The approach is designed to mitigate retinal degeneration caused by the immune response, targeting the damage where it is most likely to occur.

Hendrik Scholl, MD — $600,000
Institute of Molecular & Clinical Ophthalmology Basel
Restoring Dormant Retinal Cell Function
Dr. Scholl is developing an optogenetic therapy to restore function to dormant cone photoreceptor cells for potentially a broad range of inherited retinal diseases. Cones are responsible for high-acuity, daytime vision, and in a certain percentage of patients, remain in a dormant state. This effort will perform late-stage preclinical studies that are required to start the first-in-human cone-based optogenetic vision restoration clinical trial. This optogenetic therapy produces a protein that makes dormant cone cells sensitive to light.

Tom Reh, PhD — $874,690
University of Washington
Enabling the Retina to Generate New Photoreceptors
Dr. Reh is developing a process to enable the human retina to grow its own new photoreceptors. Thus far, he has used a small molecule to sprout photoreceptors from Muller glia in mice. The TRAP project is for evaluating the approach in a large animal.

Paul Yang, MD, PhD — $900,000
Oregon Health & Science University
Advancing a Pharmaceutical Therapy for Retinitis Pigmentosa
Dr. Yang is evaluating the drug mycophenolate as a therapy for multiple forms of retinitis pigmentosa and related conditions. Already approved by the FDA for inflammatory conditions, mycophenolate has been shown to reduce the accumulation of a molecule called cyclic guanosine monophosphate (cGMP). While cGMP is an important messenger molecule for converting light into electrical signals in the retina, too much of it is toxic and causes retinal degeneration.

Mahdi Farhan, MD — $999,700
Usher 3 Initiative
Advancing Small-Molecule for Usher Syndrome 3A (USH3A) in Preparation for Clinical Trial
Dr. Farhan is completing pre-IND toxicity studies to advance a novel small-molecule therapy for USH3A into a Phase 1 clinical trial. The emerging drug works by stabilizing the misfolded USH3A protein (clarin-1) and enabling it to better move to its target location in retinal cells, thereby striving to preserve structure and function.

Rob Collin, PhD — $1,414,167
Radboud University
Development of RNA Therapies for Stargardt Disease
Dr. Collin is developing antisense oligonucleotides (AON) – tiny pieces of DNA – to mask splicing mutations in ABCA4, the affected gene in people with Stargardt disease. The AONs target mutations in RNA, the genetic messages used to build proteins for a cell's health and proper functioning.

Program Project Awards

Alberto Auricchio, MD — $2,500,000
Fondazione Telethon
MicroRNA-based Therapy for Inherited Retinal Diseases (RetMir)
Dr. Auricchio and a team of co-investigators, through their program called "RetMir," are identifying microRNAs and microRNA antagonists or 'sponges' that have a neuroprotective effect on the retina in order to provide a one-fits-all, mutation-independent therapeutic approach for a broad range of inherited retinal degenerations.

William Beltran, VMD, PhD — $2,400,000
School of Veterinary Medicine - University of Pennsylvania
Penn Large Animal Translational & Research Center
The PENN Large Animal Model Translational and Research Center plays a critical role in bridging basic science and the testing of new therapies in clinically relevant canine models by supporting the research conducted by inherited retinal disease investigators affiliated with the facility and Foundation Fighting Blindness-sponsored scientists from other institutions. The grant is focusing on therapy development and evaluation of canine models for a number of IRDs and will include studies to prepare for clinical trials for Best disease and retinitis pigmentosa gene therapies.

BEACON STORY:

Reaching for the Stars

by Lauren Reeves

Before Brendon Cavainolo was even born, his mom, Lisa Pleasants, was familiar with inherited retinal diseases, knowing she could potentially pass a retinal disease onto her future child. Lisa's two brothers and cousin were diagnosed with X-linked juvenile retinoschisis (XLRS), so she saw first-hand how much it affected their lives.

At only three months old, Brendon had genetic testing and was diagnosed with XLRS. Once Brendon was old enough to communicate, a doctor determined he was legally blind.

"The day Brendon was diagnosed was one of the worst days of my life," says Lisa. "I wanted to do everything in my power to make sure Brendon didn't struggle in his life."

At a young age, Brendon was fascinated with space and expressed interest in becoming a rocket scientist, so Lisa enrolled him in space camps during the summer. Brendon also had some powerful role models growing up, like his uncle, who was also affected by XLRS and was an electrical engineer for NAVAIR (Naval Air Systems Command).

In high school, Brendon continued to excel in math and stayed busy with extracurriculars like teaching himself and playing the guitar. For two years, Brendon was also a member of his high school's cross-country team, developing a support system with his teammates, who offered him rides to and from practice.

Brendon and his mom, Lisa, standing in front of the Casey Eye Institute of Oregon Health & Science University.

"My diagnosis never made me feel that different growing up. It didn't really hit me until high school when all my friends started getting their driver's licenses; that's the only time when I felt like I was left out."

Brendon in his high school graduation photo playing a guitar.

"My diagnosis never made me feel that different growing up," says Brendon. "It didn't really hit me until high school when all my friends started getting their driver's licenses; that's the only time when I felt like I was left out."

In college, at the University of Central Florida (UCF), Brendon joined a group called the Students for Exploration of Space, an engineering-based organization, and similarly, he built a support system with this group and was the treasurer for two years.

Now 22 years old, Brendon graduated with a bachelor's degree in Aerospace Engineering from UCF in May 2021 and begins a PhD program in the fall of 2021.

Brendon also recently applied and was awarded the prestigious National Science Foundation's Graduate Research Fellowship, which recognizes and supports outstanding graduate students by providing financial support for three years of graduate studies. Past fellows include numerous Nobel Prize winners, former U.S. Secretary of Energy Steven Chu, and Google founder Sergey Brin.

"Brendon has never let his diagnosis get in his way," says Lisa. "Your child's diagnosis doesn't define them; in fact, I think it can help them become a stronger person and succeed even more." Regardless of Brendon's outstanding accomplishments in his life so far, he and his mom have stayed informed on research and seek treatment options for his XLRS.

In fact, in 2015, Brendon began participating in a natural history study at Casey Eye Institute of Oregon Health & Science University in Portland, Oregon, thanks to a recommendation from Dr. Sandeep Grover at the University of Florida. The objective of this study, funded by the Foundation, was to evaluate subjects with XLRS in a clinical setting and gather data on disease progression. Brendon visited every six months for two years, each visit consisting of three-to-four-hour two-day testing performed, which looked at the natural history of his rare disease. The results of this study were used to design future gene therapy clinical trials.

Brendon and his VisionWalk team all wearing their red "Team Brendon" t-shirts.

When this two-year evaluation was complete, Brendon was 18 years old and became eligible for a clinical trial at Mass Eye and Ear. But in pre-enrollment, they discovered Brendon had a pressure issue in his eye, so he was not eligible to participate.

"I still have a lot of hope for technology, with the way the tech industry is advancing at a shockingly fast pace," says Brendon. "I think technology is going to transform treatments for diseases like mine."

To continue their search for treatment and remain optimistic, Brendon and Lisa connected with Dr. William Hauswirth at the University of Florida, who recommended they get involved with the Foundation Fighting Blindness. At that time, the Foundation's Jacksonville Chapter and VisionWalk were just beginning to grow, and Lisa has been a vital member and contributor for the last 14 years.

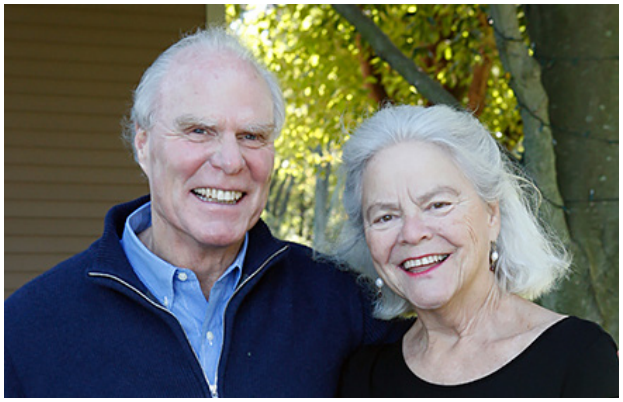
"At Foundation events, I stuck Brendon in the spotlight at a young age, but it's really helped him gain a lot of self-confidence," says Lisa. "For many years, he's been there to help the Foundation, but the Foundation has helped him grow quite a bit too."

Through Brendon's adversity and an abundance of hard work to get to where he is today, he's learned that having a community is essential to living a happy and successful life.

"In going through any type of vision loss, it's important to keep an open mind and make lots of friends," says Brendon. "Developing a support system has been so helpful in my journey with vision loss, but you also have to be your own advocate, so learn how to stand up for yourself as well."

Brendon standing in front of a rocket.

The Strength of the Foundation is Through Our Communities



Gordon and Lulie Gund.

Cindy and Barbara taking a walk to celebrate the Fall Virtual VisionWalk in San Diego.

From the beginning, Lulie was there. She was a voice of inspiration in the early days of Gordon Gund's fight against retinitis pigmentosa and always present as a guiding hand, helping him realize his vision... for the Foundation Fighting Blindness.

As the Foundation enters its 50th year, we begin Lulie's Next Chapter for Light and Vision.

The Foundation Chapter network, originally conceived by Lulie herself, brings people together across the country to join in fighting blinding retinal diseases. Our family of volunteers and leaders stand up for those impacted by blinding diseases. From day one, they share the latest research and advancements and provide access to local resources to guide individuals through their journey. And together, they celebrate the Foundation's mission.

We are winning thanks to the dedication of our Chapters focusing on three key areas: education, resources, and revenue. Through informative sessions, Foundation staff, community, and national experts provide research information, low vision resources, and outreach to our local retinal specialists and eye care professionals. To continue accelerating our mission, each community is invited to be a stakeholder by participating in our fundraising events.

The Foundation Fighting Blindness Chapter vision is to bring communities together to end blinding diseases. To join a Chapter, visit: FightingBlindness.org/Chapters

Virtual VisionWalks

2021 was another year of pivoting events to keep the Foundation's mission moving forward in new ways. The Foundation continued with new virtual experiences to bring together and engage our communities. The VisionWalks were all virtual this year, raising over **$2.8 million**.

Hope from Home: A Night to Save Sight

On February 28, 2021, the Foundation kicked off the 50th anniversary celebrations with Hope from Home, which included celebrity emcee Saturday Night Live alum Kevin Nealon, musical performances by Lachi and Charlie Kramer, and the presentation of our highest research honor – the Llura Liggett Gund Award – to Jean Bennett, MD, PhD. More than 600 households logged in to experience the Foundation's first-ever virtual gala, chaired by Foundation board members and leaders Jonathan Steinberg, MD, and Alice Cohen, MD. Thanks to the generosity and support of the event committee, sponsors, attendees, and donors, over **$600,000** was raised.

Microsoft Scramble for Sight Golf Tournament

On August 5, 2020, the Microsoft Scramble for Sight Golf Tournament was held at the Arrowhead Golf Course in Littleton, Colorado. The tournament was sold out, raising over **$135,000**, and was a wonderful day of food, games, and hearing heartfelt stories from participants as they shared their experiences of vision loss, and in some cases, regaining their sight. Thank you to the dedicated and long-time tournament chairs, Scott Burt and Sherri Kroonenberg, who made this event a major success.

Night for Sight: Celebrating 50 Years of the Foundation

On June 24, 2021, the Foundation celebrated its 50th anniversary and its new Beacon Society through Night for Sight, a virtual gala presented by Two Blind Brothers. Thanks to the support of so many, Night for Sight raised over **$570,000** for critical research finding treatments and cures for blinding diseases. This event was led by co-chairs and Foundation board directors Jason Ferreira and Evan Mittman and featured phenomenal entertainment from music icon and nine-time Grammy winner Sheryl Crow. Through Night for Sight, six spectacular leaders were recognized as part of our inaugural Beacon Society: Allegiance Retail Services, Peter and Gretchen Crowley, Porky Products, Glenn Sblendorio, Scott Sennett, and John Sharko.

Steve McGuire, Danny Vaninger, Lizzie Samson, and Olivia McGuire on the putting green at the Microsoft Scramble for Sight.

Snapshot of Our Grants and Awards

The Foundation's Scientific Advisory Board is composed of preeminent, international clinical and scientific leaders in ophthalmology and vision research. These new research awards are selected through a rigorous review process conducted by the Foundation's Scientific Advisory Board.

July 2020 – June 2021 (Fiscal Year 2021)
In FY2021, new research awards:

$13,900,000

120
Letters of intent (LOI) reviewed across all funding opportunities

52
Applications reviewed across all funding opportunities

5
Foundation-sponsored or co-sponsored meetings and workshops attended

3
Study sections conducted

FY21 Funding Opportunities

- **Clinical Innovation Award** (folded into Individual Investigator Research competition)
- **Diana Davis Spencer Clinical Research Fellowship Award** (3 Apps)
- **Career Development Award** (8 Apps)
- **Individual Investigator Research Award** (53 LOI/17 Apps)
- **Free Family Initiative in AMD** (24 LOIs/ 4 Apps)
- **Program Project Award** (8 LOIs/4 Apps)
- **Translational Research Acceleration Award** (35 LOIs/15 Apps)
- **Resource Award** (1 App)

New Awards

- **1 Clinical Innovation Award** ($300,000)
- **1 Diana Davis Spencer Clinical Research Fellowship Award** ($65,000)
- **2 Career Development Awards** ($1,500,000)*
- **4 Individual Investigator Research Awards** ($1,200,000)
- **1 Free Family AMD Award** ($600,000)
- **1 Program Project Award** ($2,500,000)
- **7 Translational Research Acceleration Awards** ($5,400,000)
- **1 Resource Award** ($2,375,000)

1 Clinical Consortium Natural History Study

The awards included in this report are those approved for funding during FY21. The start date of these awards varies and could be either FY20 or FY21.

**This includes 2 Career Development Awards approved in FY21 but budgeted for FY22.*

Developments of the My Retina Tracker® Registry and Genetic Testing Program

The Foundation's no-cost genetic testing program and the My Retina Tracker Registry for people with inherited retinal diseases (IRDs) continued to evolve and expand impressively during the fiscal year 2021. Since its launch in 2017, more than 10,000 people with IRDs have received no-cost genetic tests through our testing partner, Blueprint Genetics, which screens for mutations in inherited retinal disease genes using a comprehensive 322-gene panel. Blueprint Genetics is committed to patient privacy and never shares personally identifying data. No-cost genetic counseling provided by InformedDNA helps patients and families understand what the testing results mean and can guide them to the research underway that is relevant to their conditions.

Approximately 19,000 people are now active participants in the Registry, which connects patients and researchers, including therapy developers recruiting for clinical trials while protecting patient privacy. The Foundation is committed to improving the member experience. This year, the first user experience survey was launched, and results from that survey are guiding the Registry team to adjust its operations and priorities.

"We have heard from our Registry members that they would like to receive more updates from the Foundation. In response, we have begun to send out more frequent news updates to our members. These updates are in addition to the targeted notifications we send—at the request of partners—to Registry members who are potential candidates for research studies, including clinical trials. It's important that members maintain an up-to-date profile so we can target communications appropriately, but also because many of our research partners are interested in learning more about the impacts of IRDs." said Todd Durham, PhD, senior vice president of clinical and outcomes research at the Foundation. "In rare diseases, patient-focused registries have become important sources of real-world data to inform the clinical development strategy."

FOUNDATION FIGHTING BLINDNESS

My Retina Tracker Registry

The Foundation greatly appreciates the support of its partners for helping drive the growth and success of both the Registry and the genetic testing program:

- Applied Genetic Technologies Corporation (AGTC)
- Blueprint Genetics
- Eloxx Pharmaceuticals
- George Gund Foundation
- InformedDNA
- MeiraGTx/Janssen
- ProQR Therapeutics
- Hope in Focus

PROFESSIONAL OUTREACH:

Empowering Eye Doctors to Deliver Hope to Retinal Disease Patients

The Professional Outreach department was established in February 2019 to educate eye care professionals around the US about the Foundation and its many resources for patients with retinal degenerative diseases. These resources include the Foundation's My Retina Tracker® Genetic Testing Program and My Retina Tracker® Registry, educational events and webinars, online research news articles, and national chapter network. The overarching goal is to help eye care professionals deliver hope and a path forward to their retinal disease patients.

The department now has more than 1,000 eye care partners, which include a variety of practices and organizations such as low vision clinics and professionals, schools of optometry, professional associations, and retinal specialists.

As a result of the COVID-19 pandemic, the department conducted much of its outreach through more than 40 webinars to partners, including four national webinars, two of which provided continuing education credits (COPE and CME) to attendees.

The Foundation's FY21 National Webinars for Eye Care Professionals Included:

"Practical Management of Inherited Retinal Disorders" (COPE/CME)

Rachel Huckfeldt, MD, PhD
Mass Eye and Ear

"Clinical Trials of Emerging Therapies for Retinal Degeneration Patients" (COPE/CME)

Alan Kimura, MD, PhD
Colorado Retina Associates

"Low Vision Resources and Rehabilitation for Retinal Degeneration Patients"

Rebecca Kammer, OD, PhD
University of California, Irvine

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California Pacific Medical Center

Denny Moyer, BS, COTA/L, SCALV,
Ensight Skills Center for Low Vision Rehabilitation

Monica Perlmutter, OTD, OTR/L, SCLV, FAOTA
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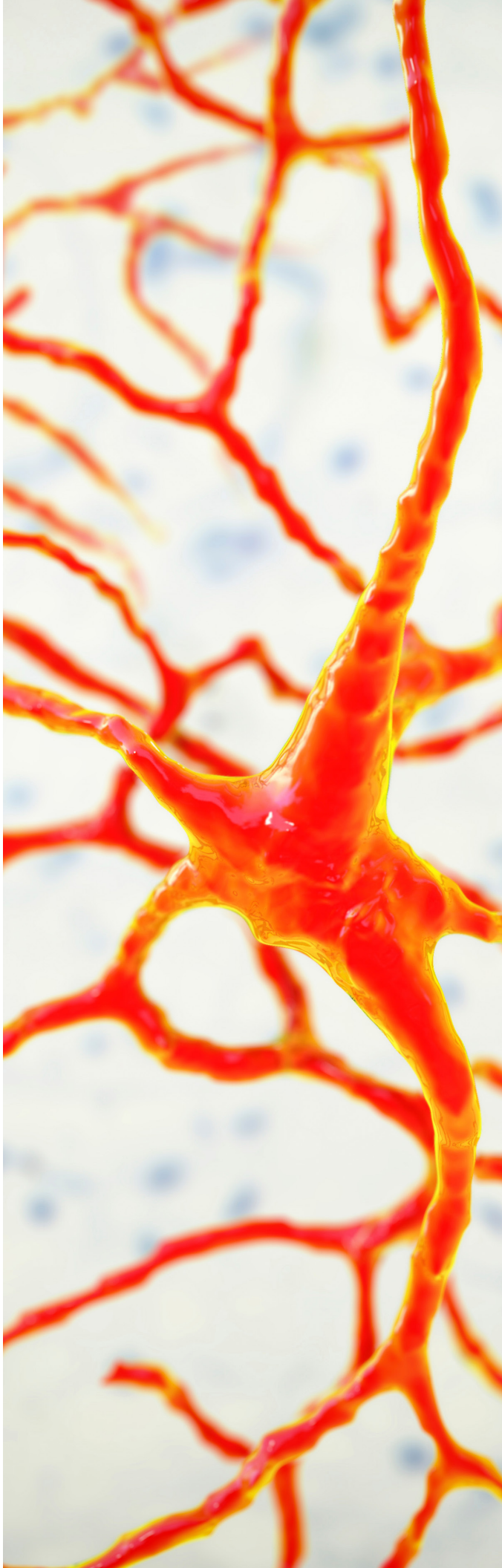
"21 Cool Research Advances for 2021"

Michelle Glaze
Foundation Fighting Blindness

Ben Shaberman
Foundation Fighting Blindness

Caesar Luo, MD
Bay Area Retina

If you are an eye care professional and want to learn more about becoming a partner, contact us at info@fightingblindness.org.



A Message from Our Treasurer

On behalf of the board and staff of the Foundation, I want to thank you for your ongoing support. Please know that the care that goes into stewarding and shepherding the funds raised by the Foundation is significant. As a contributor myself, someone with a rooted interest in the outcome of these efforts, I couldn't be more proud of how we handle your donations.

I am pleased to present the statement of activities and financial position for the fiscal year ending June 30, 2021. The total revenue came in just shy of $41 million this fiscal year. We deployed $21.4 million towards research, spent $1.7 million on public health and education, and incurred fundraising and management expenses of $8.5 million. We had net assets of approximately $165 million, which included almost $157 million that is committed to scientific research through donor-restricted funding for grants, endowments, board restrictions, and the RD Fund.

I joined the Foundation when my young son, Jack, was diagnosed with a blinding disease. My wife, Leslie, immediately got online and found that the Foundation's St. Louis Chapter was having an upcoming meeting. We went, and we were immediately inspired. Two weeks later, I was on a plane to the VISIONS conference, where I was introduced to Gordon Gund. It doesn't take knowing him very long to be convinced that Gordon is the guy you want to team up with.

We've been actively engaged ever since—VisionWalks, Chapter meetings, Dining in the Dark dinners, conferences — and we are pleased to help raise money and help bring attention to this cause.

I joined the board of directors about four years ago, and in this, my first year as treasurer, I can report to you that the Foundation, despite the challenges of the pandemic, outperformed its budget and is fiscally healthy with operational resources to keep us strong in the event of additional challenges. That's a testament to our staff and leadership and their ability to pivot to virtual events — not to mention the commitment of our donors.

In addition to my service with the Foundation, I previously served on the board of the RD Fund. You can read more about the RD Fund later in this report, but you can rest assured that their work is amplifying the effects of your donations.

We are at a time in history when we are literally on the cusp of curing the diseases that cause blindness. The magnitude of that accomplishment and the impact on individuals, their families, and society is massive. When we achieve it — and we are going to — it will be as exciting to be a part of as anything we can imagine.

Given the progress made since Jack's diagnosis — made possible in part thanks to this incredible Foundation — we are confident that something will develop that will help him retain his existing vision and possibly improve, or even restore, his sight. That's the holy grail of what we're all searching to find. And none of it would be possible without the help of supporters like you. Thank you for all you do.

Sincerely,

Jason Morris
Treasurer

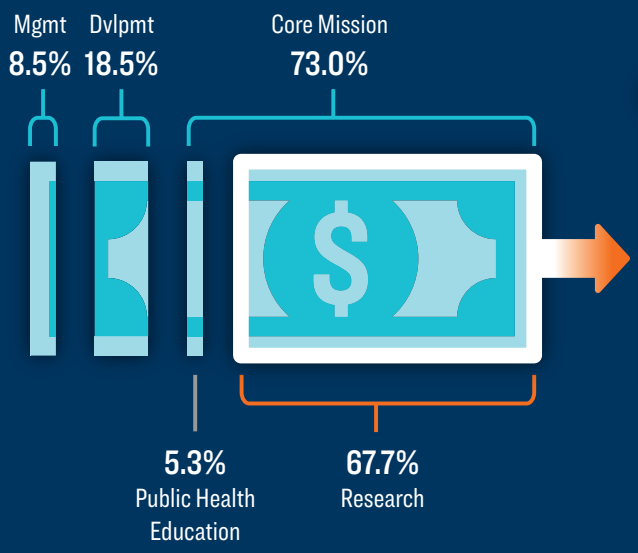
Statement of Activities

	June 30, 2021	June 30, 2020
Revenue and Support		
Contributions	$17,474,000	$24,979,000
Special events revenue, net of direct expense	4,741,000	5,234,000
Bequests	3,998,000	1,222,000
Other revenue	14,604,000	3,304,000
Total Revenue	**$40,817,000**	**$34,739,000**
Expenses		
Research	$21,377,000	$20,909,000
Public Health Education	1,683,000	1,730,000
Management	2,697,000	1,959,000
Fundraising	5,836,000	7,235,000
Total Expenses	**$31,593,000**	**$31,833,000**
Total Change in Net Assets	**$9,224,000**	**$2,906,000**

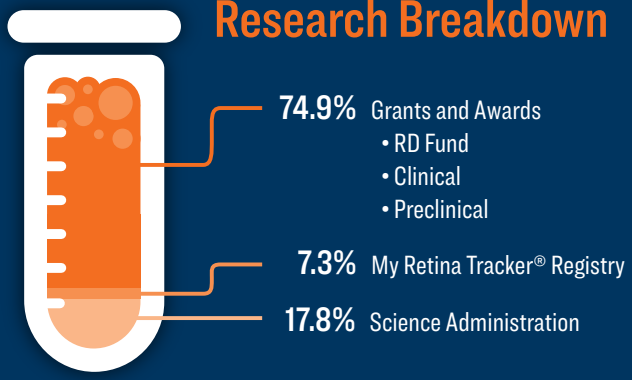
Statement of Financial Position

Assets		
Cash and investments	$131,966,000	$120,538,000
RD Fund Investments	21,507,000	11,038,000
Pledges receivable, net	13,673,000	28,416,000
Other assets	3,844,000	2,096,000
Trusts and other funds	8,704,000	7,293,000
Fixed assets, net	949,000	1,154,000
Total Assets	**$180,643,000**	**$170,535,000**
Liabilities		
Accounts payable and accrued liabilities	$2,937,000	$2,305,000
Research grants payable	11,728,000	11,655,000
Deferred revenues	266,000	7,000
Liabilities under trusts and other funds	618,000	688,000
Total Liabilities	**$15,549,000**	**$14,665,000**
Net Assets	**$165,094,000**	**$155,870,000**
Total Liabilities and Net Assets	**$180,643,000**	**$170,535,000**

Donation Allocations



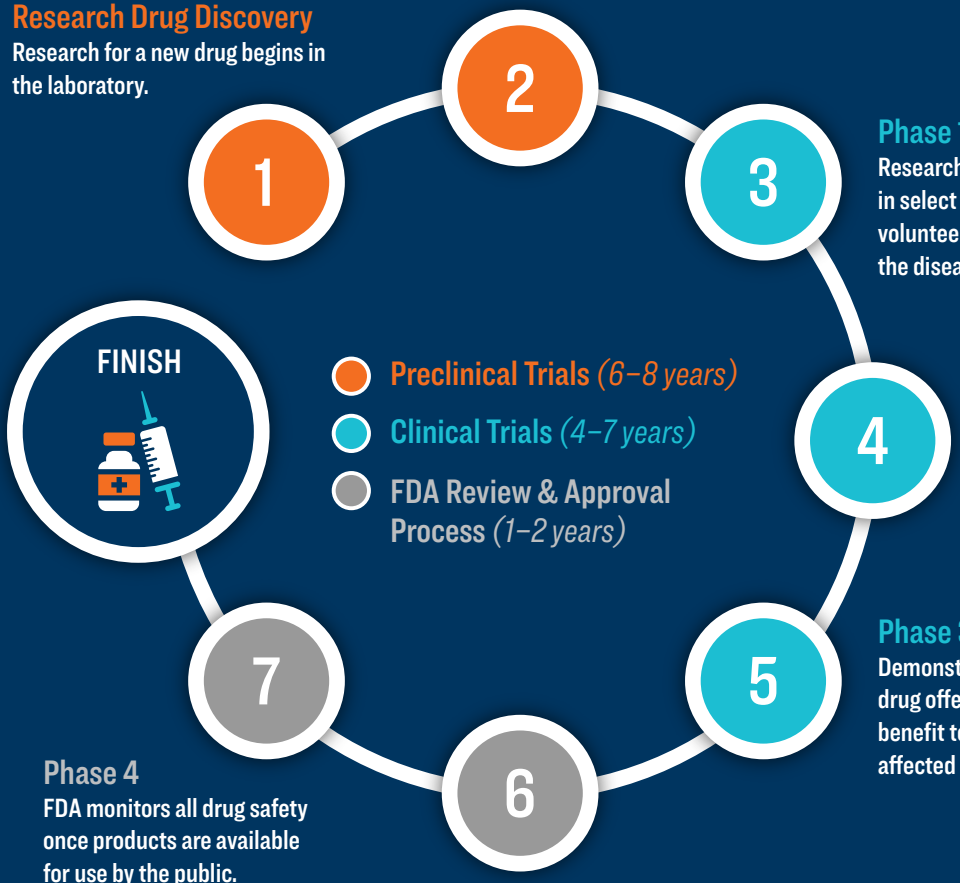
Research Breakdown



Research & Drug Development Process for Rare Diseases

Research Drug Discovery
Research for a new drug begins in the laboratory.

Translational / Non-Clinical
Drugs undergo laboratory and animal testing to answer basic questions about safety and an acceptable dose range.



- Preclinical Trials *(6–8 years)*
- Clinical Trials *(4–7 years)*
- FDA Review & Approval Process *(1–2 years)*

Phase 1
Researchers test a new drug in select group of healthy volunteers or people with the disease of interest.

Phase 2
Administration of drug to small group to provide indication of the benefits and risks of the drug.

Phase 3 "Pivotal Trials"
Demonstrate whether or not drug offers a clear treatment benefit to larger population of affected individuals.

FDA Review
FDA thoroughly examines all submitted data to approve or not to approve drug .

Phase 4
FDA monitors all drug safety once products are available for use by the public.

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The Foundation's National Trustees are leadership-level volunteers who support the Foundation's fundraising, organizational development, and volunteer recruitment efforts.

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The Strategic Council was created with the goal of connecting young professional leaders to drive the next wave of innovation at the Foundation Fighting Blindness from a unique and diverse vantage point, leveraging various backgrounds and talents. They work directly with the Foundation leadership and management to collaborate and problem-solve strategic challenges and opportunities being faced today—preparing themselves to be the next generation of leaders for the Foundation.

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The Foundation Fighting Blindness Scientific Advisory Board is comprised of the world's leading retinal experts who provide insight on research and clinical advancements and review research grant applications.

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RD FUND

RDFund.org

The RD Fund is a 501(c)(3) not-for-profit subsidiary of the Foundation Fighting Blindness that invests in cutting-edge companies focused on inherited retinal diseases. These investments further the research and generate even more funds that are poured right back into furthering our mission. The RD Fund portfolio companies leverage over five times the RD Fund's invested capital from outside traditional venture capital firms.

To learn more about the RD Fund, visit: **RDFund.org**

A Message from the RD Fund Chair

I began working with Foundation Fighting Blindness nearly two decades ago. I am not a scientist, and there is no blindness in my family. I came aboard because I wanted to continue working with Foundation co-founder Gordon Gund. After years of helping him manage his investments, I knew how passionate Gordon is about so many things — especially the mission of the Foundation. His passion is infectious.

But that passion doesn't stop with Gordon. That same passion is evident anywhere you look across the Foundation, including the RD Fund (Retinal Degeneration Fund). I have served as chair of the RD Fund, the venture arm of the Foundation, since its creation three years ago. And it's one of the most exciting and rewarding things I've ever done.

The RD Fund has an independent board of directors comprised of investors, executives, and clinicians — brilliant minds from all across the worlds of science and business. They all share this passion for bringing every possible resource to bear in the quest to wipe out these diseases. The RD Fund leverages the full weight of the Foundation's knowledge and resources, including the Scientific Advisory Board, the Clinical Consortium, and the My Retina Tracker Registry — along with an experienced and skilled management team.

In 2018 the fund was launched with an initial investment of $72 million. That enabled us to fund 10 promising companies, all with exceptional CEOs. In the fiscal year that ended June 30, 2021, we had our first successful exit of one of the first 10 companies we funded. But that early success did not breed arrogance. Our team remains hungry, and we are determined to win this fight.

The companies we invest in are all doing stellar work, but as I don't pick favorite children, I can't pick one favorite investment or piece of science. It is the portfolio and the family that I am most excited about. There is measurable success in the field now, in no small part because of the work being done by the Foundation Fighting Blindness and fueled by you and others.

You can trust that your gift to the Foundation is supercharged by the RD Fund. And you can trust that the passion the determination, and commitment that helped to start the Foundation 50 years ago is alive and well today. Thank you for your trust and your investment.

With gratitude,

Warren Thaler
RD Fund, Chair

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In its first three years, the RD Fund has invested in promising companies, including:

Atsena is developing gene therapy products including a clinically staged candidate for one of the most common causes of blindness in children.
—Pat Ritschel, MBA, CEO

CheckedUp is the only, physician-founded, specialty healthcare technology platform designed to engage patients, caregivers, and physicians in the waiting room, exam room, and at home.
—Richard Awdeh, MD, CEO

Lookout Therapeutics is a RD Fund spin-out company founded with a leading venture capital group with significant expertise in gene therapy and rare diseases.
—Paul B. Manning, CEO

Nacuity is developing an anti-oxidant treatment for retinitis pigmentosa and other related indications including Usher syndrome.
—Halden Conner, CEO

Nayan is developing variant-agnostic therapies to treat inherited retinal diseases such as forms of retinitis pigmentosa.
—Milind Deshpande, PhD, CEO

ProQR is developing RNA therapies to treat inherited retinal diseases including Leber congenital amaurosis and Usher syndrome.
—Daniel de Boer, MBA, CEO

SparingVision is developing a novel gene therapy approach for the treatment of inherited retinal diseases such as retinitis pigmentosa.
—Stéphane Boissel, MBA, CEO

Stargazer Pharmaceuticals is an ophthalmic biotech company focused on developing a novel visual cycle modulator to slow the accumulation of toxic retinoids in the eye for Stargardt disease.
—David Meek

Vedere Bio (I and II) is a biotech company utilizing novel ocular gene therapy technologies to develop a pipeline of vision restoration and vision preservation therapies for underserved indications.
—Cyrus Mozayeni, MD, MBA, CEO

The RD Fund 1 Investment Financial Summary:

Assets	
Funds Committed to Date	$53,783,506
Reserves for future funding for RD Fund portfolio companies	19,185,632
Funds for Future Investments	0
Total Assets	**$72,969,138**

The RD Fund 2 Investment Financial Summary:

Assets	
Funds Committed to Date	$10,000,000
Reserves for future funding for RD Fund portfolio companies	20,000,000
Funds Available for Future Investments and Reserves	11,100,000
Total Assets	**$41,100,000**

RD Fund Leadership and Management

Board of Directors

Warren Thaler, MBA
Chair,
RD Fund

David Brint
Chair,
Foundation Fighting Blindness

Anthony P. Adamis, MD

Eugene de Juan, MD

Jacque Duncan, MD

Adrienne Graves, PhD

Kelly Lisbakken

Jonathan Steinberg, MD

Management Team

Benjamin Yerxa, PhD
Chief Executive Officer

Rusty Kelley, PhD, MBA
Senior Vice President,
Investments and Alliances

Jason Menzo
Chief Operating Officer

Peter Ginsberg
Executive Vice President,
Corporate Development,
Chief Business Officer

Claire M. Gelfman, PhD
Chief Scientific Officer

VENTURE PHILANTHROPY:

The Secret Weapon for Unlocking Biomedical Research's Full Life-Changing Potential

by Gordon Gund & Paul Manning

More than a year into the COVID-19 pandemic, there has been much reflection around "lessons learned" across all sectors. In the biomedical research space, we've seen science meet the urgent need for safe and effective vaccines at miraculous speed to contain the spread of the virus. The mRNA technology used in some of those vaccines has broad implications for future treatments for a variety of other viruses, cancers, and diseases and is a clear indication of how far science has evolved in a short period of time. Imagine what treatments and cures could be unlocked — with the necessary funding.

In the United States, public funding for basic research has long come from the National Institutes of Health, but the U.S. government lags other advanced economies in the amount of funding it provides for the translational research required to convert basic science into tangible patient treatments. And while more public funding for biomedical research at the critical clinical trial stage is essential, it is going to take public, private, and philanthropic dollars to ensure that biomedical research into promising treatments and cures doesn't wither on the vine. Federal programs such as the Cancer Moonshot, state-level initiatives like the California Institute for Regenerative Medicine, and promising legislation aimed at providing private-sector loans to companies developing novel treatments for disease and disability are all helpful — but still leave a funding gap. There needs to be a third leg to stabilize those public- and private-sector efforts, and we believe that third leg is philanthropy.

As successful entrepreneurs and venture investors, we see our donations as investments in the mission of the nonprofit organizations we support. We each have a personal connection to the mission of the Foundation Fighting Blindness: one of us has experienced loss of sight from retinitis pigmentosa as a young adult, and the other has raised two sons with vision impairment caused by Stargardt disease. Based on our personal experiences, we have a keen understanding of what it is like to be a patient or have a loved one waiting for life-changing treatments to become available.

For fifty years, thanks to the generosity of donors, the Foundation Fighting Blindness has successfully funded research in pursuit of treatments and cures for the entire spectrum of inherited retinal diseases (IRDs) and dry

age-related macular degeneration (AMD), which together affect more than two hundred million people globally. Yet, more needs to be done. The key discoveries made in labs need to make it into the hands of industry-led therapy developers to conduct clinical testing and win FDA approval. But a gap in funding often prevents this progress, and in this case, the science is now outpacing the funding.

To bridge this funding gap, the Foundation Fighting Blindness created the Retinal Degeneration Fund (RD Fund), a nonprofit, pure-play venture philanthropy investment vehicle designed to help accelerate the technical aspects of the organization's mission and advance its financial goals. Our respective family foundations contributed significant capital to launch the fund, which allowed us to be more involved in the organization's work by funding highly visible activities in biotech startups and spinouts. We've taken concepts and techniques from our venture capital finance and business management experience and applied them to our philanthropic goals of accelerating the progress on treatments and cures, while positioning the organization for long-term sustainability.

Launched in late 2018 with $72 million under management, the first fund is now 90 percent committed, with nine investments plus reserves. This invested capital has attracted an additional $400 million in capital to date from institutional co-investors and has produced its first exit with the sale of Vedere Bio to Novartis for $280 million, enabling the organization to plug a financial gap in its long-range science spending plan and roll over significant funds to seed Fund 2.

We take comfort in knowing that the venture philanthropy model already has been successfully scaled by the Bill & Melinda Gates Foundation, the Cystic Fibrosis Foundation, and the Juvenile Diabetes Research Foundation, just to name a few. One key element is to manage it professionally and deliberately; one cannot just wander into biotech equity investing without experience, deep scientific know-how, and world-class advice and oversight. The RD Fund has an independent board of directors with expertise spanning retinal biology, clinical ophthalmology, finance, and entrepreneurship, and the board works closely with an executive management team with significant operational, strategic, and leadership experience. Importantly, the fund is able to rely on an international scientific advisory board and leverage the organization's patient registry and clinical consortium. In other words, the brain trust of the Foundation Fighting Blindness and its venture arm have the collective scientific and business acumen to best determine what is or is not an investible mission-related opportunity.

We are encouraged by venture philanthropy's ability to reap a return to be re-invested in furthering an organization's mission, especially in times of economic uncertainty. Most important, our experience has demonstrated that jump-starting the pipeline for treatments and cures through venture philanthropy holds real promise as a viable, scalable approach for addressing other underserved diseases impacting so many.

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FightingBlindness.org
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