Together, we’re winning.

2020 ANNUAL REPORT
“At first, the progress was slow, searching for where to begin. Soon the small wins compounded, and we learned more and grew stronger.”
Dear Friends,

As CEO and Board Chair of this life-changing Foundation, David and I extend our sincere thanks to you, our partners in the fight for vision. Thanks to a dedicated staff, exceptional volunteer leadership, world-class scientists, and donors like you, we are the world’s leading organization searching for and finding preventions, treatments, and cures for retinal degenerative diseases.

The 2020 Annual Report highlights our collective progress toward that mission — a mission that you make possible.

As we reflect on the past year, we must acknowledge the global pandemic that upended the world in early 2020. COVID-19 has impacted our ability to gather in person and as a result, caused many of our fundraising efforts to change course. But with a lot of hard work, and as a result of generous friends like you, we’ve managed to close out the fiscal year on a positive note.

While the primary purpose of this annual report is an accounting of activity up to June 30, 2020, as always, we are looking ahead to new victories, and more treatments and cures. There have been five decades of fundraising, research, and trial and error that have led us to this point, and all the wins along the way are why we can look with confidence to the future.

Our report on the 2020 fiscal year is guided by three key areas: ensuring expertise in the field, fueling the research pipeline, and winning the fight for vision.

We Are the Experts

Every year, the Foundation Fighting Blindness deploys significant resources for the development of treatments and cures. We are leading a global effort in collaboration with the top experts in the field and supporting nearly 80 labs across the world.

The Foundation didn’t start this way. In 1971, we struggled to attract researchers to the study of inherited retinal diseases. But we were driven by the commitment of our chairman and co-founder Gordon Gund and his late wife Lulie, and countless others who were passionate about the mission. Together, with donors like you, we fought to recruit a generation of researchers.

At first, the progress was slow, searching for where to begin. Soon the small wins compounded, and we learned more and grew stronger. Ten years ago, only a few clinical trials were underway. Today, the Foundation has funded the research that made many of the more than 40 clinical trials possible.

The recent growth in this area of research is exponential. We’ve identified 300 genes, and the only thing standing in the way of finding cures is adequate funding.

At the heart of this progress is a robust engine of resources that includes the Clinical Consortium, the Scientific Advisory Board, and the My Retina Tracker Registry.

The Clinical Consortium is a group of 39 clinical sites around the world, all working together to study how inherited retinal diseases progress so they can develop outcome measures for efficient clinical trials. The Scientific Advisory Board is a brain trust of the world’s leading retinal experts who provide the Foundation insight on research and clinical advancements and review research grant applications.

To ensure that we maintain our roster of experts in the field, we fund programs like The Diana Davis Spencer Clinical Research Fellowship Award. This initiative provides funding for doctors in clinical fellowships. The program helps us increase the number of clinician-scientists with the expertise and commitment to provide clinical care to patients with inherited retinal disease.

In support of all our work is our My Retina Tracker Registry. Over 16,000 people are now active participants in the Registry. The Registry is a precious resource for patients and families and an invaluable tool for researchers.

In addition, the Open Access Genetic Testing Program offers no-cost genetic testing and counseling to help patients and families understand the genetic cause of their disease, what those results mean, and guide them to research that is relevant to their conditions.

While the growth of the Registry has been impressive, we need to do more. Since there are upwards of 200,000 people in the U.S. with an inherited retinal disease and more than four million worldwide, there is no shortage of patients to engage. To continue advancing the science, we need as many of those people to take part as possible.
Hannah, a recent LUXTURNA success story.

At seven years old, Hannah had never seen a lightning bug in the summer or a star in the sky. But after her treatment with LUXTURNA, Hannah can now play outside at night with her brothers and has a new bright future in store. Hannah’s story is the kind of winning that the Foundation has been striving toward for nearly a half-century. We have an enormous pipeline of treatments in development, with the potential to create countless more stories like Hannah’s. And it’s all thanks to the generosity of supporters like you. But it’s not enough. The science is there, waiting for us. We continue to need funds to put the science to work. You and others like you know the impact we can make. You light a fire under us. It’s no longer a question of the science, it’s a question of funding the science.

We can’t stop now. Yes, it’s a heavy lift, but with your help, we firmly believe we can defeat these diseases in our lifetime.

As we prepare to celebrate our 50th anniversary in 2021, David and I ask for your continued support. Because together, we will win.

With our sincerest thanks,

David Brint, Chairman

Benjamin R. Yerxa, PhD, Chief Executive Officer

The Promise of the Research Pipeline

Our work is shaped every day by what we call the Pipeline to Vision. Engagement leads to investment, which funds research, which ultimately leads to treatments, cures, and a victory for vision.

The first step in generating the necessary funding for a robust research pipeline is engaging with current supporters while also inviting new people to join our mission. Engagement starts at the local level, with over 40 volunteer-led chapters across the country that increase awareness, provide support, and raise money for families affected by retinal diseases. As in years past, we held multiple annual VisionWalk events, which are essential in bringing in new supporters.

Because of the pandemic, our first-ever National Virtual VisionWalk was held in June. And despite the shift in execution, it raised more than $1.2 million.

Another significant strategy for fueling the research pipeline is the Retinal Degeneration Fund (RD Fund)—made possible by donors who contributed to the Gordon and Llura Gund Family Challenge. Launched in 2018 as a not-for-profit venture arm of the Foundation, the RD Fund helps accelerate life-changing outcomes for people with inherited retinal diseases through direct investment in companies developing therapies.

These investments further the research and generate even more funds that are poured right back into accelerating our mission. With more than $72 million under management, the RD Fund is not only leveraging the resources and expertise of the Foundation, but also attracting significant capital from outside investors.

Together, We’re Winning

Thanks to you, the Pipeline to Vision is full like never before. It’s producing potential treatments and cures. It’s producing hope. It’s producing a victory for vision.

All this investment, all this research, all this work—it’s paying off in unprecedented ways. Thanks to you, we’re winning. Early proof of these wins is LUXTURNA®, the first FDA-approved gene therapy for the eye or any inherited disease. Later in this report, along with more information about VisionWalks, clinical trials, and all our activity, you can also read about Hannah, a recent LUXTURNA success story.

At seven years old, Hannah had never seen a lightning bug in the summer or a star in the sky. But after her treatment with LUXTURNA, Hannah can now play outside at night with her brothers and has a new bright future in store. Hannah’s story is the kind of winning that the Foundation has been striving toward for nearly a half-century. We have an enormous pipeline of treatments in development, with the potential to create countless more stories like Hannah’s. And it’s all thanks to the generosity of supporters like you.

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With our sincerest thanks,

David Brint, Chairman

Benjamin R. Yerxa, PhD, Chief Executive Officer
There has been remarkable growth in clinical development for retinal degenerative disease treatments in 2020. Foundation funding continues to play a leading role in advancing the field, especially in moving emerging therapies into and through clinical trials. More than 40 clinical trials are underway for potential treatments for retinal diseases.

**This fiscal year’s research highlights include:**

**Nacuity’s Emerging Anti-Oxidative Therapy Moves into Clinical Trial for RP associated with Usher Syndrome**

Fort Worth-based Nacuity Pharmaceuticals launched a Phase 1/2 clinical trial in Australia for NPI-001, an oral anti-oxidant designed to slow vision loss in people with retinitis pigmentosa (RP) and RP associated with Usher syndrome. The Foundation is investing $7.5 million in NPI-001 development through its RD Fund.

**Foundation Invests $3 million in Atsena Therapeutics, New Company Developing GUCY2D-LCA1 and MYO7A-USH1B Gene Therapies**

The gene therapy for Leber congenital amaurosis (LCA) caused by mutations in GUCY2D is in a Phase 1/2 clinical trial at the University of Pennsylvania. A dual-vector gene therapy for Usher syndrome type 1B, which is caused by mutations in MYO7A, is in pre-clinical development. Atsena Founder and Chief Scientific Officer Shannon Boye, PhD, University of Florida, is the pre-clinical developer for the company’s emerging gene therapies. The Foundation provided significant lab funding to Dr. Boye for
her pioneering gene therapy work in MYO7A and other retinal genes.

**jCyte Reports Promising Results for Phase 2b Clinical Trial of its Cellular Therapy for RP**

jCyte, a biotech company based in Newport Beach, California, announced that its emerging cellular therapy for people with retinitis pigmentosa (RP) and related diseases has performed encouragingly in a Phase 2b clinical trial. The company plans to launch a Phase 3 clinical trial for the treatment in 2021. The goal of the therapy is to preserve or potentially restore some vision in people with RP and related conditions, independent of the mutated gene causing the disease.

**AGTC Planning Phase 2/3 Clinical Trial for XLRP Gene Therapy**

Applied Genetics Technology Corporation (AGTC), a developer of gene therapies for rare diseases, is planning to launch a Phase 2/3 clinical trial in the first quarter of 2021 for its emerging X-linked retinitis pigmentosa (XLRP) gene therapy for people with mutations in the gene RPGR. AGTC is also expanding its Phase 1/2 XLRP gene therapy trial. In January 2020, AGTC reported a favorable safety profile and evidence of vision improvement for the XLRP gene therapy in the Phase 1/2 trial. The Foundation funded critical lab research to make this gene therapy clinical trial possible.

**MeiraGTx and Janssen Pharmaceuticals Report Promising Interim Results from its Phase 1/2 Clinical Trial for XLRP Gene Therapy**

MeiraGTx and Janssen Pharmaceuticals report stable or improved retinal sensitivity for five of seven participants — those in the low and intermediate dose groups — in a Phase 1/2 clinical trial for its X-linked retinitis pigmentosa (XLRP) gene therapy. The therapy is for XLRP caused by mutations in the gene RPGR. Thanks to the encouraging interim results, the company is planning a Phase 3 trial for the treatment.

**ProgStar Study Identifies Potential Endpoint for Clinical Trials of Emerging Stargardt Disease Treatments**

An international research team funded by the Foundation recently showed that microperimetry may be an effective outcome measure for clinical trials of emerging Stargardt disease therapies. A microperimeter is a device that measures retinal sensitivity in the macula, the central region of the retina. The results of the study were published online in JAMA Ophthalmology.

**Interim Results Released for USH2A RNA Therapy Clinical Trial**

ProQR Therapeutics, an RNA therapy developer in the Netherlands, has announced three-month, interim Phase 1/2 clinical trial results for QR-421a, an emerging treatment for people with Usher syndrome type 2A and non-syndromic retinitis pigmentosa (RP) caused by mutations in exon 13 of the USH2A gene. The company reported that the treatment was well tolerated and caused no serious adverse events. Also, 25 percent of patients receiving the treatment demonstrated improved vision.

**First Patient Receives Emerging CRISPR Therapy in Clinical Trial for LCA 10**

Clinical researchers at Casey Eye Institute, Oregon Health & Science University (OHSU), have dosed the first patient with an experimental CRISPR/Cas9 therapy in the BRILLIANCE Phase 1/2 clinical trial for people with Leber congenital amaurosis 10 (LCA 10). The emerging treatment targets a specific mutation (c.2991+1655A>G in Intron 26) of the gene CEP290. The trial is the first time an emerging CRISPR therapy was administered inside the human body.

**Iveric Bio’s Therapy Slows Retinal Degeneration in Phase 2b Trial for Dry AMD**

Zimura®, an emerging therapy for people with advanced dry age-related macular degeneration (AMD), slowed progression of geographic atrophy, the lesions signifying retinal degeneration and potentially vision loss, in a Phase 2b clinical trial. Results were reported after 12 months of treatment. Patients will be treated in the Phase 2b for a total of 18 months.
The Foundation Around the Nation

In the first half of the fiscal year, our Fighting Blindness family across the country came together to raise funds, increase public awareness, and provide support for their communities. After the COVID-19 pandemic struck, our entire community, including our staff, board members, trustees, committees, volunteers, chapters, and team captains from across the country had to pivot to keep the Foundation’s mission moving forward in new ways. The Foundation created new virtual experiences to keep meaningful community engagement momentum going. In the end, wherever we are, we are stronger together – as a community.

The 13th Annual Twin Cities VisionWalk hosted over 670 attendees that came out to help further our mission. These Vision-Walkers, sponsors, volunteers, and other supporters raised over $244,760 to advance the Foundation’s mission.

Youth Chair Maeva Murphy cuts the ribbon with the help of her mom and Dr. Bhavsar of The Retina Center at the start of the Twin Cities VisionWalk as other walkers cheer.

The 14th Annual Colorado VisionWalk was a picture-perfect day with over 500 participants who formed 30 teams and raised $214,320, a new all-time high. The Colorado VisionWalk has exceeded its goal for the past five years and aims to keep the streak going!

Pictured right: Colorado VisionWalkers at the starting line with a balloon arch and the venue in the background.
The 12th Annual **Boston VisionWalk** was celebrated at Artesani Park in Brighton, MA. Over 400 walkers showed their support of the Foundation, with special appearances by famous Boston sports’ mascots, raising over $226,000 to find cures for retinal degenerative diseases.

The 17th Annual **Taste for Sight** in New York, New York featured a selection of wines, beer, spirits, and seasonal fare in support of the urgent mission of the Foundation Fighting Blindness. Taste for Sight highlighted 21 local breweries and wineries, auctioned off over 50 exclusive items and experiences, and in total raised over $178,000 for the Foundation.

The 19th Annual **Microsoft Scramble for Sight** golf tournament held on August 7, 2019, hosted 130 golfers and 80 volunteers and families at Arrowhead Golf Club in Littleton, Colorado. Honorary guest Olivia Klinefelter and her father shared her LUXTURNA story with attendees, which has restored her vision, going from 20/800 to 20/50 today. The sponsors, golfers, and auction raised an all-time high of $143,974, which brings the event’s 19-year total to over $2.2 million.

The Foundation made the tough decision to pivot on our approach to VISIONS 2020 that was planned for Minneapolis, MN, to an all-virtual event. The new virtual approach was a great success, and we hope everyone that participated gained insight to the most recent advances in retinal research and experienced a sense of community with the Fighting Blindness family. Our mission is your vision! All Virtual VISIONS 2020 presentations were recorded and are available to watch at: FightingBlindness.org/recorded-sessions
Hannah’s Bright Future Ahead

At seven years old, Hannah had never seen a lightning bug in the summer or a star in the sky. But after her treatment with LUXTURNA, Hannah can now play outside at night with her brothers and has a new bright future in store.

Pictured left: Hannah outside in the woods hiking with her two brothers, Matthew (left) and Jacob (middle).

Hannah Reif was diagnosed with a visual impairment when she was just three months old.

At two weeks old, Hannah’s mom, Amy, took her to a routine wellness checkup. The pediatrician asked Amy if Hannah was making eye contact with her, and she immediately responded, “yes” because she knew it was the right answer. But after putting more thought into it, Amy honestly wasn’t sure. After that appointment, Amy and Hannah’s father, Chris, kept a closer watch on her eyes.

Over the next few months, Amy and Chris realized that Hannah, in fact, wasn’t making eye contact with her. Hannah would only look out the window at the sun or a lamp. Amy even saw what looked like Hannah’s eyes bounce up and down, and that’s when she started to realize something wasn’t right. Within days of Hannah’s next checkup, she was referred to a pediatric ophthalmologist and then sent to Children’s Hospital of Philadelphia (CHOP) for an
electroretinography (ERG) test. That same day, the doctor told Amy that Hannah’s ERG results came back flat, diagnosing her with Leber congenital amaurosis (LCA). Shortly after, Hannah received genetic testing to confirm she did have LCA with mutations in the RPE65 gene. Their retinal specialist told them about a clinical trial happening right there at CHOP for a gene therapy that Hannah could potentially be a candidate for one day.

“Learning about the ongoing clinical trial as soon as Hannah was genetically tested was really promising for us,” says Amy. “We were thrilled knowing that the trial was going on right where we lived, and it gave us so much hope. At that early point in Hannah’s diagnosis, it’s all you can hold onto.”

Amy recalls immediately feeling overwhelmed by Hannah’s diagnosis, but having the Foundation Fighting Blindness as a resource helped her to feel more informed and prepared for Hannah’s future. The Reif family attended their first Foundation VISIONS conference when Hannah was only seven months old. This experience involved connecting with other parents and individuals affected by rare diseases like Hannah’s, which helped them to feel a part of the Fighting Blindness family.

“All you hear initially is how rare of a disease your daughter has,” says Amy. “I didn’t know anything about vision loss, but to have an opportunity to meet others affected by diseases so similar to hers so early on, it was really wonderful.”

The Reif family has also participated in the Philadelphia VisionWalk every year since Hannah was one year old.

Over the next several years, Amy and Chris continued to hold onto hope and brought Hannah to CHOP for her annual checkups, hearing of the clinical trial progress each time.

Two weeks after Hannah turned seven years old, on December 19, 2017, Amy and her family heard the news that LUXTURNA® had become FDA approved. They knew the trial had been so successful, but the day LUXTURNA was an FDA-approved gene therapy felt unreal.

Amy waited a couple of weeks before reaching out, but then at the beginning of January, she contacted Spark Therapeutics, the company developing the treatment. Over the next several months, the Reif family waited until CHOP became an approved treatment site.

“It was a no brainer that we would have the procedure done at CHOP,” says Amy. “Although it was obviously so exciting, it was still pretty terrifying too.”

Hannah was the first patient to receive LUXTURNA at CHOP after its FDA approval. Dr. Albert M. Maguire performed Hannah’s surgery, who was part of the team that brought the genetic therapy to fruition.

On July 10, 2018, Hannah had her first surgery on her left eye, and Amy and Chris could immediately tell Hannah could see better.

“She came downstairs the next morning for breakfast, sat down at the table, and turned on her desk lamp like she always had every morning,” recalls Amy. “But she immediately pushed the light away and started to cry because the light
was too bright. She had never had a sensitivity to light; she always needed more light before.”

Hannah’s vision was cloudy at first, but only six days after her first surgery, Hannah saw a star for the first time in her life.

Two weeks later, after Hannah’s second surgery on her right eye, new discoveries continued. Hannah could now see the buttons on the microwave, the hot and cold controls for air in the car, the water line in her glass, and more.

“She’d be filling up a glass of water and say, “Hey, I can see a water line in my glass now,” when before she had to stick her finger in the glass to know if it was full,” says Amy. “As a mom, I wanted to cry every time she discovered something new that she could see, but Hannah just stated it, very casually, and never really got excited about it, she just took it all in stride.”

Before Hannah’s surgery, the Reif’s were always mindful of the lighting when visiting new places with Hannah. Amy and Chris would carry a portable desk lamp, in case the lighting was too dim for Hannah. But now, the entire family has been able to explore new places, like Disney World, and not once have to worry about how the lighting would be for Hannah.

Hannah now has the freedom to run around and play outside with her brothers and friends, even once the sun goes down. Her new favorite summer activity is catching lightning bugs, which she couldn’t even see before, let alone be able to navigate outside in the dark.

Thanks to LUXTURNA, Hannah’s world is now brighter and clearer than ever, giving her the ability to navigate all on her own, giving her confidence she never had before.

“It’s wonderful as a parent to see that Hannah can now navigate independently and that daily tasks aren’t a struggle anymore,” says Amy. “It’s been an amazing experience, and we feel so fortunate for this entire process.”
2020 Research Investments

This fiscal year, the Foundation committed $6.5 million for 15 new research projects for inherited retinal diseases. These new grants include development of CRISPR/Cas9 gene-editing treatments, new disease models, and a retinal regeneration therapy. The Foundation currently funds a total of 84 research grants. The projects were selected from 135 proposal submissions made by investigators. Listed below are some of the significant research developments made by the Foundation during the past fiscal year.

Neural Regeneration through Stimulation of Muller Glia
Tom Reh, PhD, University of Washington

Tom Reh, PhD, is continuing his innovative research in a treatment that empowers the retina for self-regeneration. While most regenerative retinal therapies involve transplantation of new retinal cells derived from stem cells, Dr. Reh’s approach would enable a diseased retina to grow new photoreceptors. The goal is to find out whether we can stimulate regeneration of new neurons in human retina from the Muller glia using the same factors that work in mice.

Homology-Independent Genome Editing for Treatment of Stargardt Disease
Ivana Trapani, PhD, Università degli Studi di Napoli “Federico II” Naples, Italy

Gene therapy development for Stargardt disease is challenging because ABCA4, the mutated gene causing Stargardt disease, is too large for most viral delivery systems. Dr. Trapani is evaluating a highly efficient CRISPR/Cas9 gene-editing technology — homology independent targeted insertion (HITI) — to address mutations in ABCA4. Her early proof-of-concept studies are being performed in a Stargardt disease mouse model and a three-dimensional retinal organoid in a dish.

ADAR-Based RNA Editing as a Potential Therapy for Inherited Retinal Degenerations
Dror Sharon, PhD, Hadassah-Hebrew University Medical Center

Many emerging therapies target mutations in our genes — our DNA. Dr. Sharon is investigating a new tool for editing RNA — the genetic messages derived from DNA which are used by our cells to produce proteins. Known as “intrinsic adenosine deaminase acting on RNA,” or ADAR, the technology specifically addresses G-to-A mutations. Dr. Sharon believes that about 30 percent of the most commonly reported IRD-causing mutations can be targeted for ADAR editing (mainly in relatively large genes such as ABCA4 and USH2A). His lab is evaluating the technology in cells and mouse models of retinal disease.
Prime Editing for Usher Syndrome Type 2A
Bence Gyorgy, MD, PhD, Institute of Molecular and Clinical Ophthalmology Basel

Dr. Gyorgy is developing a gene correction strategy known as prime editing, a novel technology that is in some ways similar to gene editing with CRISPR/Cas9, but potentially more precise and efficient. Instead of cutting the double strands of DNA, it nicks the DNA, which may be a preferred approach to correcting single-letter mutations. Dr. Gyorgy is developing a prime editing treatment to insert the missing G nucleotide into retinal cells with the relatively common USH2A mutation del2299G. In order to facilitate translation of this concept to the clinic, he will evaluate the therapy in human retinal explants, human-engineered retinal organoids, and humanized mouse models. If the approach is successful, it may be applied to other mutations and IRDs.

Creation of New Models of Inherited Retinal Disease in Pigs
Maureen McCall, PhD, University of Louisville

Though rodents are often used as models for inherited retinal diseases (IRDs), they are limited in recapitulating human IRDs because they have few cones, the retinal cells that enable humans to perceive details and colors, and see in lighted settings. Pigs do have cones and are easy to engineer to model IRDs in humans. Dr. McCall is creating a slow degeneration pig model of RP (RHO-P23H) as well as a pig model of Stargardt disease. The models will be useful for evaluating emerging therapies for clinical trials.

Generation and Characterization of a Pig Model of Retinitis Pigmentosa (EYS mutation)
Hemant Khanna, PhD, University of Massachusetts Medical School

Mutations in the EYS gene are the second most common cause of autosomal recessive RP in humans. However, the intact EYS gene is absent and/or disrupted in many animals, including mice and rats. The gene is present in pigs, so Dr. Khanna and his colleagues are developing a pig model of EYS-associated RP, which will be useful in understanding the effects of EYS mutations and testing emerging EYS therapies.

Large Animal Model of Usher Syndrome Type 1B
Martha Neuringer, PhD, Oregon Health & Science University

One of the biggest challenges in developing therapies for the vision loss caused by Usher syndrome is that the existing animal models (namely rodents) for the condition don’t have vision loss. Using gene-editing techniques, Dr. Neuringer and her colleagues are developing a large animal model of Usher syndrome type 1B, which is caused by mutations in the gene MYO7A. She is characterizing the resulting syndrome from birth onward, including changes in the retina and auditory system. She will also perform an initial test of the feasibility of using this model to evaluate dual-vector gene therapy to maintain retinal function and vision. In a separate grant, the Foundation is funding Shannon Boye, PhD,
University of Florida, to develop a dual-vector MYO7A gene therapy for Usher 1B.

**Developing a Large Animal Model of Stargardt Disease**

Botond Roska, MD, PhD, Institute of Molecular and Clinical Ophthalmology Basel

Stargardt disease affects the fovea, the central part of the retina that is responsible for high visual acuity. However, rodents don’t have foveae, so they don’t serve as good models for Stargardt disease. Dr. Roska is developing a Stargardt disease model in marmosets, which do have foveae. His model will have the mutation c.5882 G>A (p.G1961E) in ABCA4 — a mutation that affects 15-20 percent of humans with Stargardt disease. The model will contribute to understanding the disease mechanisms and help researchers to evaluate not only small molecules but also precision gene editing tools to restore affected ABCA4 protein function.

**Penn Large Animal Translational and Research Center**

William Beltran, VMD, PhD, School of Veterinary Medicine-University of Pennsylvania

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 (IRD) investigators affiliated with the facility and Foundation Fighting Blindness-sponsored scientists from other institutions. This 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 (RP) gene therapies.

**Advancement of Ellipsoid Zone Intensity as a Surrogate Biomarker for Photoreceptor Structure**

Joseph Carroll, PhD, Medical College of Wisconsin

Dr. Carroll and his team are using optical coherence tomography (OCT) to develop and improve validated markers of retinal structure, including the ellipsoid zone (EZ) band which correlates with the population of healthy photoreceptors in patients’ retinas. The investigators are studying EZ band as a marker for photoreceptor health. Validated markers developed in the study will enable clinical researchers to more effectively evaluate emerging therapies in clinical trials for a number of conditions, including retinitis pigmentosa, Stargardt disease, achromatopsia, and choroideremia.

**Assessing the Function of Individual Cells in Patients with Inherited Retinal Diseases**

Robert Cooper, PhD, Marquette University

Ophthalmoscopes are the devices that eye doctors use to examine patients’ retinas during office visits. Dr. Cooper is developing a highly sensitive ophthalmoscope, which incorporates adaptive optics, to visualize retinas at the cellular level. Determining the rate of disease progression often requires years of clinical examination. Even then, changes in disease presentation are often subtle and difficult to reliably
assess. By developing a more sensitive ophthalmoscope for measuring disease progression, eye doctors can provide patients with more timely and precise assessments of their retinal health.

**Scrutinizing Protein Complex Assembly in Photoreceptor Connecting Cilia**  
Ronald Roepman, PhD, Radboud University Medical Center

Photoreceptors, the long sensory cells that make vision possible, are comprised of two primary parts: the outer segment, which senses light, and the inner segment, which produces the proteins that are shuttled into the outer segment so that the photoreceptor processes light. A small channel between the inner and outer segment is called the connecting cilium and is often a bottleneck for protein assembly and trafficking in retinal diseases such as Leber congenital amaurosis, Usher syndrome, Bardet Biedl syndrome, and retinitis pigmentosa. Dr. Roepman will be creating a “retina in a dish” to recreate/model the bottleneck and investigate ways to overcome it to save vision.

**Ocular Perfusion in Retinal Degeneration**  
Giacomo Calzetti, MD, Institute of Molecular and Clinical Ophthalmology Basel

Research to date has not determined the contribution of retinal blood flow to degeneration in retinal diseases. Dr. Calzetti is developing new imaging techniques and methods to assess blood flow in USH2A and ABCA4 patients to facilitate a deeper understanding of the pathophysiology of inherited retinal diseases.

**Characterization of Biomarkers for Cystoid Macular Edema in Retinitis Pigmentosa**  
Cristy Ku, MD, PhD, Oregon Health & Science University

Current treatment options for cystoid macular edema in retinitis pigmentosa (RP-CME) are limited. Dr. Ku is investigating the mechanisms behind RP-CME through analyzing biomarkers of inflammation in the aqueous humor and plasma in patients with RP-CME. Dr. Ku’s research seeks to improve the clinical management of RP-CME by identifying novel therapeutic targets.

**Optimizing Gene Therapy for Choroideremia: Redefining Cellular Targets, Treatment Windows and Outcome Measures**  
Erin O’Neil, MD, University of Pennsylvania

Ongoing clinical trials to assess gene therapy for choroideremia have shown modest effectiveness. Dr. O’Neil is exploring additional retinal cell targets that may improve gene therapy outcomes. Additionally, she will study the early natural history of choroideremia to identify the best time to intervene with gene therapy early in disease.
Growth and Enhancements for Genetic Testing Program and My Retina Tracker® Registry

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 fiscal year 2020. Since its launch in 2017, more than 8,000 people with IRDs have ordered no-cost genetic tests through 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 personal 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 16,000 people are now active participants in the Registry, which connects patients and researchers, including therapy developers recruiting for clinical trials. During this year, the Registry was migrated over to a new robust platform, which greatly improved usability for patients and families, including those who are blind or have low vision.

“We are very pleased to be helping people with an inherited retinal disease receive a clearer diagnosis guided by genetic testing and supported by genetic counseling to better understand what the results mean. A diagnosis is also helping people with IRDs get on the radar screen of clinical investigators,” said Brian Mansfield, PhD, executive vice president of research and interim chief scientific officer at the Foundation. “With more than 31 gene or mutation-specific clinical trials underway for emerging therapies, there are increasingly more opportunities for people to participate in the research.”

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
- Blueprint Genetics
- Eloxx Pharmaceuticals
- InformedDNA
- MeiraGTx/Janssen
- ProQR Therapeutics
- George Gund Foundation
- Sofia Sees Hope
Professional Outreach: Educating Eye Care Professionals about Mission and Patient Resources

The Foundation’s professional outreach team was established in February 2019 to build and cultivate relationships with eye care professionals and others who serve the blind and low vision community. The team educates professionals about patient resources, the My Retina Tracker Registry and Open Access Genetic Testing Program, clinical research, and emerging therapies, as well as Foundation-hosted educational meetings. Through these relationships, the team seeks to increase the number of patients who are referred to the Foundation for support, resources, and hope. The team has built a network of more than 700 facilities and doctors for collaboration and patient referrals. The team maintains ongoing relationships with: 35 key opinion leaders (KOLs), 12 schools or colleges of optometry, 15 large retina practices (with 20+ doctors), 35 Foundation chapter-directed professional outreach volunteers (located in 28 markets within the country), and 10 state/regional/national organizations.

In May 2020, the professional outreach team organized and hosted the Foundation’s first-ever continuing education course (CME/COPE) for eye care professionals including ophthalmologists, retinal specialists, and optometrists. The presentation on inherited retinal diseases, genetic testing, and clinical trials was delivered as a live webcast by Dr. Jacque Duncan, University of California, San Francisco, and chair of the Foundation’s Scientific Advisory Board. More than 200 eye care professionals participated in the live webcast. A recorded version of the course is available on the professional outreach section of the Foundation’s website.

The professional outreach team also hosted a genetic testing webinar in June 2020 in collaboration with Blueprint Genetics and Informed DNA, attracting nearly 100 eye care professionals. The 90-minute workshop provided information on ordering no-cost genetic testing and counseling online through the Open Access Genetic Testing Program. Throughout 2020, the team hosted more than 35 webinars and live presentations to members of the eye care community. These were tailored for various audiences, including KOLs, eye care organizations, patient groups, schools/colleges of optometry, Foundation chapter volunteers, low vision specialists, and rehabilitation professionals. The team also participated in multiple conferences hosted by organizations such as Envision, VisionServe Alliance, and The Optometric Retina Society.
Clinical-Trial Pipeline

Inherited Retinal Diseases and Dry AMD: 42 Trials (select)

Visit ClinicalTrials.gov for more details and trial contact information. This document is for informational purposes only. Information is subject to change, and its accuracy cannot be guaranteed.

GENE THERAPIES.......................... PROGRESS
Achromatopsia (CNGB3) – AGTC........................................ Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx................................ Phase 1/2
Achromatopsia (CNGA3) – AGTC........................................ Phase 1/2
Achromatopsia (CNGA3) – STZ EyeTrial................................ Phase 1/2
AMD (Dry) – Gyroscope.................................................. Phase 1/2
Choroideremia (REPI) – 4DMT......................................... Phase 1/2
Choroideremia (REPI) – BioGen......................................... Phase 3
Choroideremia (REPI) – Spark........................................... Phase 1/2
Choroideremia (REPI) – Tubingen Hosp............................. Phase 2
LCA (GUCY2D) – Atsena.................................................. Phase 1/2
LCA and RP (RPE65) – MeiraGTx...................................... Phase 1/2
LCA and RP (RPE65) – Spark............................................. Phase 2/FDA Approved
RP (PDE6B) – Horama..................................................... 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 (RLBP1) – Novartis....................................................... Phase 1/2
RP (PDE6B) – STZ EyeTrial............................................. Phase 1/2
Retinoschisis (RS1) – NEI............................................... Phase 1/2
X-linked RP (RPGR) – AGTC......................................... Phase 1/2
X-linked RP (RPGR) – MeiraGTx...................................... Phase 1/2
X-linked RP (RPGR) – BioGen........................................ Phase 2/3

CELL-BASED THERAPIES.......................... 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/b
RP, Usher (retinal progenitors) – iCyte.............................. Phase 2/b
RP, Usher (retinal progenitors) – ReNeuron........................ Phase 2
Stargardt (RPE) – Astellas............................................... Phase 1/2

MOLECULES, PROTEINS, AONS.................. PROGRESS
AMD-dry (C3 inhibitor) – Apellis......................................... Phase 3
AMD-dry (CB inhibitor) – Ionis............................................ Phase 2
AMD-dry (C5 inhibitor) – Iveric Bio.................................... Phase 2
LCA (CEP290, AON) – ProQR.......................................... Phase 2/3
LCA (CEP290, CRISPR) – Editas........................................ Phase 1/2
RP (RHO, AON) – ProQR.................................................... Phase 1/2
Stargardt disease (emixustat) – Acucela.............................. Phase 3
Stargardt disease (deuterated vt 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
Usher syndrome (NACA-anti-oxidant) – Nacuity................... Phase 1/2
Usher syndrome 2A (AON) – ProQR.................................... Phase 1/2

Scientific Advisory Board Year in Review

July 2019 – June 2020 (Fiscal Year 2020)
In FY2020, new research awards from applications reviewed:

$6,500,000

50 Applications reviewed across all funding opportunities
135 Letters of intent (LOI) reviewed across all funding opportunities
9 Science sessions during Virtual VISIONS 2020
3 Study Sections

- Clinical Innovation Award
  (Reviewed within the Individual Investigator Research competition)
- Diana Davis Spencer Clinical Research Fellowship Award (5 Apps)
- Individual Investigator Research Award
  ($99 LOI/31 Apps)
- Free Family Initiative in AMD
  (27 LOIs/4 Apps)
- Non-rodent Large Animal Resource Award
  (9 LOIs/8 Apps)
- Translational Research Acceleration Award
  (1 App)
- Facility Award
  (1 App)
- Ted and Elaine Welp Enhanced Career Development Program

*The awards included in this report are those awarded and initiated between July 1, 2019 and June 30, 2020. This aligns with the Foundation’s fiscal year.*
A Message from Our Treasurer

On behalf of the Foundation Fighting Blindness staff and leadership, I want to thank you for your support. As the leading nonprofit funder of inherited retinal disease research, fiscal responsibility is at the core of everything we do. Donors, supporters, and partners of the Foundation trust us to make the right investments to find preventions, treatments and cures. We are thoughtful and deliberate about how we put those funds to work—and we know none of it would be possible without you.

I am pleased to present the statement of activities and financial position for the fiscal year ending June 30, 2020. Revenue and support came in just shy of $35 million. For FY 2020, we deployed $21 million towards research, spent $1.73 million on public health and education, and incurred fundraising and management expenses of $9.2 million.

As of June 30, 2020, we had Net Assets of approximately $156 million, which included $144 million that is committed to scientific research through donor restricted funding for grants, endowments, board restrictions, and the Retinal Degeneration Fund (RD Fund).

Reviewing the 2020 fiscal year, you have to consider the impact of the COVID-19 pandemic. There is no doubt we have seen a negative impact on our fundraising. With cost-cutting and additional philanthropic investment, we still managed to increase our net assets by approximately $3.9 million over the previous fiscal year.

In summary, I am happy to report that the Foundation’s ability to fund vision-saving research is as strong as ever. This financial position of strength is a tribute to the passion of our donors and the importance of the mission.

On a personal note, I am stepping down in my role as treasurer after ten years, and I’m leaving the position in the capable hands of Jason Morris. My history with the organization and the mission goes back much longer than my 10-year tenure as treasurer. My daughter, Elizabeth, was diagnosed with LCA at age 5. Today she’s 36. She graduated law school at the top of her class and is a mom to a 7-year-old and a 10-year-old. She’s lost most of her vision now, but remains as strong and positive as ever. I am beyond proud of her.

I am also beyond proud of the life-changing progress we have made as a Foundation. Your investments have helped fill the Pipeline to Vision. There are so many more treatments and cures waiting to be discovered. Together we are stronger, and we can achieve our goal of making blinding degenerative retinal diseases a thing of the past.

With my sincere gratitude to you for trusting us with your resources,

Haynes P. Lea, Treasurer
Statement of Financial Position

June 30, 2020 | June 30, 2019
--- | ---
**Assets** | |
Cash and investments | $131,577,000 | $132,958,000
Pledges receivable, net | $28,415,000 | $26,255,000
Other assets | $2,096,000 | $1,787,000
Trusts and other funds | $7,293,000 | $7,450,000
Fixed assets, net | $1,154,000 | $1,329,000
--- | ---
Total Assets | $170,535,000 | $169,779,000

**Liabilities** | |
Accounts payable and accrued liabilities | $2,305,000 | $1,589,000
Research grants payable | $11,655,000 | $13,981,000
Deferred revenues | $17,000 | $316,000
Liabilities under trusts and other funds | $688,000 | $929,000
--- | ---
Total Liabilities | $14,665,000 | $16,815,000

**Net Assets** | |
| $155,870,000 | $152,964,000
**Total liabilities and net assets** | $170,535,000 | $169,779,000

---

Statement of Activities

June 30, 2020 | June 30, 2019
--- | ---
**Revenue and Support** | |
Contributions | $24,979,000 | $37,179,000
Special events, net of direct | $5,234,000 | $7,035,000
Bequests | $1,222,000 | $5,185,000
Other revenue | $3,304,000 | $5,527,000
--- | ---
Total revenue | $34,739,000 | $54,926,000

**Expenses** | |
Research | $20,909,000 | $21,947,000
Public Health Education | $1,730,000 | $1,689,000
Management | $1,959,000 | $2,079,000
Fundraising | $7,235,000 | $7,080,000
--- | ---
Total Expenses | $31,833,000 | $32,795,000

**Total change in net assets** | |
| $2,906,000 | $22,131,000

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Year Ahead: Target Spending Allocations

Research, Including
Grants and Investments ............70%
Fundraising ..........................20%
Public Health Education ............3%
Administration .......................7%
The Foundation Fighting Blindness maintains a network of chapters across the country. The chapter network has three areas of focus: resources, education and revenue. They hold seminars and meetings that provide information on research, low vision resources, and other helpful industry topics and raising revenue to fund our cutting-edge research.

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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- Princeton, NJ
  - Llura Gund President

**NORTHEAST REGION**
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  - Michael Montagnese President
- Princeton, NJ
  - *Llura Gund passed away on Sunday, March 15 at her home in Princeton, NJ*
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The RD Fund
When I explain the work of the Foundation Fighting Blindness and its mission of ending inherited retinal diseases that cause blindness, it’s hard not to gleam with pride. But when you layer on top of that the venture arm of the Foundation, the Retinal Degeneration Fund, and its success in just two short years—it’s a story of monumental significance. The Retinal Degeneration Fund was created in 2018—thanks to donors who contributed to the Gordon and Llura Gund Family Challenge—to help accelerate life-changing outcomes for people with retinal degenerations through direct, mission-related investments in therapeutic companies. It is a first-of-its-kind fund that transforms dollars into investments in cutting-edge-companies that are all focused on one mission—accelerating the science of saving vision.

The RD Fund has an independent board of directors comprised of investors, executives, and clinicians. 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.

As a donor, you are a part of the Foundation family—and by extension—the RD Fund. Allow me to share three RD Fund highlights:

1) The RD Fund helps the Foundation keep pace with research made possible by you. Because of the research funded by you through the Foundation, we can truly say that we’re winning—beating each inherited retinal disease is now only a matter of time and money. In fact, science has made such great advances that it sometimes appears to outpace our ability to fund it. That’s why we created the RD Fund. Based on a model known as “venture philanthropy,” this not-for-profit venture arm of the Foundation started with $72 million under management and, in just two short years, has experienced measured performance and advancements in the research.

What if we could build on the work of the Foundation Fighting Blindness and receive a return on our investments by achieving our mission, to accelerate the science saving vision, and a financial return at the same time? Thanks to generous investors and the Retinal Degeneration Fund, there are no more “what ifs” only “what’s next?”
2) The RD Fund is an innovative pioneer and an impactful partner. 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 IRDs. These investments further the research and generate even more funds that are poured right back into furthering our mission. The RD Fund has also successfully attracted capital from other venture investors with an average of five times outside capital funding portfolio companies to date. In addition to providing funding, the RD Fund provides invaluable ongoing technical and strategic support and guidance to the Foundation.

3) The RD Fund is hyper-accelerating the fight against blinding retinal diseases. We focus on therapeutic companies with late pre-clinical to clinical-stage programs. The initial allocation is typically $2-5 million per initial investment. As of June 30, 2020, we had invested in eight promising companies led by exceptional CEOs—all with returns going back to the Foundation to further support its mission. And perhaps most importantly, these companies take the early-stage research breakthroughs from the lab to the clinic at a pace that could not be accomplished with traditional philanthropy.

What follows is a snapshot of the RD Fund, its companies, and its highlights. The bottom line is this—the Foundation Fighting Blindness has helped to prove that these diseases can be defeated. And with the support of innovative resources like the RD Fund, we’re making your dollar go further than ever before.

Together—with you, our team, and with investors at the RD Fund—we’re winning.

Sincerely,

Warren Thaler, MBA
Chairman, RD Fund

Pictured right:
Warren Thaler, MBA
Chairman, RD Fund
In its first two years, the RD Fund has invested in promising companies, including:

SparingVision – developing a novel gene therapy approach for the treatment of IRDs such as retinitis pigmentosa.
Stephane Boissel, MBA, CEO

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

Nayan – developing variant-agnostic therapies to treat IRDs such as forms of retinitis pigmentosa.
Milind Deshpande, PhD, CEO

ProQR – developing RNA therapies to treat IRDs including Leber congenital amaurosis and Usher syndrome.
Daniel de Boer, MBA, CEO

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

Vedere – developing gene therapy products to restore functional vision to patients who have suffered vision loss from IRDs.
Cyrus Mozayeni, MD, MBA, CEO

Stargazer – an ophthalmic biotech company focused on the development of a novel treatment option for Stargardt disease.
Gary Sternberg, MD, MBA, CEO

CheckedUp – specialty healthcare technology platform designed to engage patients, caregivers, and physicians in the waiting room, exam room, and at home.
Richard Awdeh, MD, CEO
The RD Fund Investment Financial Summary:

Total RD Fund Investment Assets: $71,969,137
Funds Committed to Date: $31,400,000
Reserves for Investments Made to Date: $22,000,000
Funds Available for Future Investments and Reserves: $18,569,137

The RD Fund Leadership

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