2019

ANNUAL REPORT

WE ARE STRONGER TOGETHER

FOUNDATION FIGHTING BLINDNESS

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A MESSAGE FROM OUR CHAIRMAN & CEO

As the world's leading organization searching for preventions, treatments and cures for retinal degenerative diseases, the Foundation Fighting Blindness plays a critical role in the fight to end blindness. We are the catalyst in funding breakthrough research and innovative science to accelerate progress. To help direct our efforts this past year, we focused on five key areas:

Increasing Investments

Through a combination of donor contributions and fundraising efforts, we reached $56 million in revenue for the year, with $47 million of those funds allocated toward current and future research support. These resources allowed us to continue funding 80 ongoing research grants, in addition to initiating 14 new research projects.

> *In addition to our annual funding activities, we launched a new major initiative to expand translational and clinical research through our Retinal Degeneration Fund (RD Fund). Launched in late 2018, this venture philanthropy fund drives emerging therapies that are moving toward, or in, clinical trials. The RD Fund is part of our long-term strategy for adapting to a rapidly changing environment where many more projects are ready for translation into human trials, while the cost of clinical research is increasing. The RD Fund has more than $70 million in initial funding to invest in companies with projects that can be in clinical testing in 18 to 24 months.*
>
> *To date, the RD Fund has committed funding to six companies – Limelight Bio, Nacuity, Nayan Therapeutics, ProQR, SparingVision and Vedere Bio – totaling $42 million in currently committed capital and reserves. It is truly inspiring to see the innovations these companies are making. The RD Fund is generating significant interest, with many exciting and worthy investment opportunities coming forth regularly. More information is available at* ***RetinalDegenerationFund.org****.*
>
> *Another pioneering initiative to fund research is the bipartisan federal legislation to fund treatments and cures for blindness and severe vision impairment. The Faster Treatments and Cures for Eye Diseases Act (H.R. 2620) would establish a five-year pilot program for the creation of "Eye Bonds" to raise up to $1 billion of funding for translational research for blinding eye diseases and conditions. While moving this legislation forward through Congress and eventually into law is a multi-year process, every individual in our community can help speed up this effort by contacting their House Representative and letting them know that you support this sight-saving legislation. Learn more about this important initiative at* ***EyeBonds.com****.*

▲ *Chief Executive Officer Benjamin Yerxa on the left and Chairman David Brint on the right.*

Collaborating with Partners

We can accelerate our results by partnering with peer organizations, community leaders and other visionaries in the field. We are honored to partner with many other collaborators, such as those highlighted below.

> *Based on an anchor donation from Dr. James Free and his wife, Carole, along with other restricted funds, we have established the Free Family AMD Research Program to provide $3 million to fund new research projects over the next five years for the development of therapies for age-related macular degeneration (AMD).*
>
> *Sofia Sees Hope, a nonprofit founded by Laura Manfre and Charles Priebe to generate awareness, raise funds for research,*

and provide outreach, support and education to those affected by Leber congenital amaurosis (LCA) and other inherited retinal diseases (IRDs), made a $100,000 donation to the Foundation to support therapy development and genetic testing.

Two Blind Brothers, a non-profit clothing company, founded by Bradford and Bryan Manning, donates 100 percent of profits to fund research through a partnership with the Foundation.

The Foundation is partnering with Fight for Sight to fund grants for veterinary post-doctoral fellows and residents in ophthalmology, particularly those investigating inherited retinal degenerative diseases, as both organizations recognize that eye and vision research often begin with animal studies.

These partnerships have enabled the Foundation to invest in cutting-edge science directed towards a variety of promising research opportunities.

Evolving and Modernizing Our Infrastructure

An area of emphasis over the past year has been on improving our data, systems and processes to drive more automation and integration. In that time, we have made multiple system and structural enhancements to the backbone infrastructure of the organization. Many of these enhancements have been behind the scenes, while others are more visible to our broader community, such as our new online fundraising platform, Classy, that is used for our VisionWalks.

The automation and efficiency improvements help provide more timely reporting for our organization, reduce administrative burdens and enable staff to focus on research, outreach and advocacy, and community engagement efforts.

While we have made tremendous progress in strengthening our infrastructure, we are committed to a continuous improvement process in which we look for opportunities to be more efficient and effective. In all cases, our goal is to improve the experience of our constituents and enable our team to do the important work of driving our mission.

Enhancing Communication

We are committed to providing valuable information to our community, expanding awareness to introduce new audiences to our mission, enhancing communications to our stakeholders and ensuring that we apply best-in-class accessibility standards to make our communications fully usable to all participants.

In January 2019, we launched our "Beacon of Light" logo and messaging. This new branding conveys how the Foundation projects a beacon of light, and hope, for those in our community. The Foundation sheds light on innovative research, community, and resources illuminating a future where we've helped to bring light to darkness.

Earlier this year, we launched a new website, ***FightingBlindness.org****. Our team is working to provide compelling new content, including research updates and "Beacon Stories" that highlight individuals in our community doing great things with their talents. This content is also shared through our social media channels, enabling us to interact directly with community members. We will continue to add content, features, and accessibility improvements to our site to make it as engaging as possible.*

We established a new communications outlet with our quarterly Insights Forum calls. The purpose of these calls is to highlight the latest developments at the Foundation and in the Fighting Blindness community. We provide a snapshot of recent strategic, scientific and research updates and conduct an open question-and-answer period. These calls also have closed-captioning available. Audio replays and transcripts are archived on our website.

Galvanizing Our Community

We are grateful for the generous support of the Fighting Blindness community who continues to engage with us each year through our research programs, events, annual giving efforts.

This year we established a Community Engagement & Professional Outreach team to offer rapid, credible and personal outreach and responses to constituents and healthcare professionals. We have constructed a comprehensive "welcome" experience for newly diagnosed patients when they and their families are first introduced to the Foundation. We are also working directly with eye care professionals to educate them on the many resources available to their patients with an inherited retinal disease.

Another area of evolution is our National Trustee Program, which is focused on building sustainable development and fundraising capacity for the Foundation. The Foundation has 146 National Trustees serving across the country. Scott Burt was recently appointed to the Board of Directors as the representative of the National Trustees. We have rolled out a new collaboration and communication plan so that Trustees will be equipped to tell our story in the field.

Established toward the end of this year, the Strategic Council is a group of young professionals with an affinity to our cause and represent the next generation of Foundation leaders. This diverse group was developed to collaborate and problem solve some of the strategic challenges and opportunities we are facing today.

PLANNING FOR THE FUTURE

As we continue to evolve the organization and prepare for future success, we have undertaken a new five-year strategic planning process. The key components of the plan include $105 million in funding for critical scientific projects and a supporting operational plan focused on making continued enhancements to our fundraising capabilities and community engagement initiatives.

This plan encompasses the full spectrum of funding programs from early translational research to clinical studies, the fundraising and revenue required to fund the highest levels possible, and the communications and education plans that enable us to be as connected as possible with our constituents.

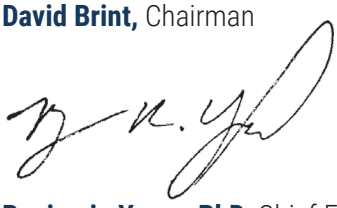
Summary

Since its formation, the Foundation has raised more than $760 million toward our search for prevention approaches, treatments, and cures for blindness caused by retinal degenerative diseases. We are excited about the many opportunities to provide funding for additional ground-breaking research at a more rapid pace and we look forward to sharing our progress.

More amazing success stories are happening every day thanks to the advancement of the research and programs fueled by your support. We are inspired and motivated by the resilience, fortitude and commitment of our community members. At the Foundation Fighting Blindness, we are driven to be a beacon of strength, a champion of courage and an advocate for hope, because we are stronger together.

Sincerely,

David Brint, Chairman



Benjamin Yerxa, PhD, Chief Executive Officer

STRONG MOMENTUM FOR VISION-SAVING RESEARCH

Clinical development for retinal degenerative disease treatments has accelerated impressively in 2019. Both current and past funding from the Foundation continue to play a leading role in advancing the field, especially in moving emerging therapies into and through clinical trials. Approximately three dozen treatments for retinal diseases (including select dry AMD treatments) are currently in human studies.

RESEARCH HIGHLIGHTS

RP Patients in ReNeuron's Cell Therapy Trial Show Further Vision Improvements

ReNeuron, a developer of cell-based therapies, has reported that patients with retinitis pigmentosa (RP) in the Phase 2a cohort of its Phase 1/2 clinical trial have demonstrated vision improvements since receiving the company's human retinal progenitor cells (hRPC). The hRPC are stem cells that have almost matured into photoreceptors, the retinal cells that make vision possible. The goal of the emerging treatment is to restore vision in people with RP and related conditions. The Foundation funded Michael Young, PhD, Massachusetts Eye and Ear, for pre-clinical and translational studies for the hRPC that helped make the ReNeuron trial possible.

AGTC Reports Promising Interim Results for XLRP and Achromatopsia Gene Therapy Trials

Applied Genetic Technologies Corporation (AGTC), a biotechnology company developing gene therapies for retinal diseases and other conditions, has reported favorable safety profiles and evidence of efficacy for participants in its gene therapy clinical trials for X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene and achromatopsia caused by mutations in the CNGA3 or CNGB3 genes. The Foundation funded critical research for these gene therapy trials. Also, AGTC used the Foundation's My Retina Tracker registry and genetic testing study to identify and recruit patients for the XLRP and achromatopsia studies.

First Patient Receives AON Therapy for LCA10 in ProQR's Phase 2/3 Clinical Trial

ProQR, an RNA therapy development company in the Netherlands, has dosed the first patient in its Phase 2/3 ILLUMINATE clinical trial for sepofarsen. The treatment, formerly known as QR-110, is designed for people with the retinal disease Leber congenital amaurosis 10 (LCA 10) caused by the mutation p.Cys998X in the gene CEP290. In the Phase 1/2 trial for QR-110, 60 percent of patients had improvements in visual acuity and their ability to navigate a mobility course. ProQR began planning the Phase 2/3 trial after reporting the positive results for the Phase 1/2.

First Patient Receives ProQR's Emerging USH2A Therapy in Clinical Trial

ProQR announced that the first clinical-trial participant has received its emerging treatment, which targets retinitis pigmentosa and Usher syndrome caused by mutations in exon 13 of the USH2A gene. The Phase 1/2 clinical trial is taking place at Retina Foundation of the Southwest in Dallas and the University of Michigan in Ann Arbor. Known as QR-421a, the treatment is intended to slow or potentially reverse vision loss. The Foundation is investing up to $7.5 million through its RD Fund to move QR-421a into and through the early stage clinical trial. QR-421a is an antisense oligonucleotide (AON) — a small piece of genetic material — designed to mask exon 13 mutations in the RNA of USH2A.

SparingVision Gets EU Funding Boost for Development of Cross-Cutting Gene Therapy

SparingVision, a French biotechnology company developing therapies for retinal degenerative diseases such as retinitis pigmentosa (RP), has received €2.5 million in funding from the European EIC Accelerator program, which is providing support for innovative, small- and medium-sized businesses in the European Union. The funding will speed clinical and regulatory development for SparingVision's emerging, cross-cutting gene therapy designed to preserve cone photoreceptors, thereby saving vision, in people with many forms of RP. The company plans to initiate a clinical trial for the treatment in the US and Europe in 2020. The Foundation has committed funding of up to €7 million for development of the gene therapy through its RD Fund, a venture philanthropy fund for treatments that are moving toward, or in, early clinical development.

Consortium Genetically Tests 40 Percent of People in Israel with IRDs

The Israeli Inherited Retinal Disease Consortium (IIRDC), a collaboration led by Hadassah-Hebrew University Medical Center in Jerusalem, has genetically tested 3,413 people with inherited retinal diseases (IRDs) from 2,420 families in Israel. The number tested is approximately 40 percent of all people affected by an IRD in the country. The Foundation provided $600,000 in funding for the project, which began in 2013. Results of the genetic testing effort were published online in the journal, Human Mutation.

FIGHTING BLINDNESS COMMUNITY SPOTLIGHTS

The ***Investing in Cures Summit*** was held on March 1–2 in Raleigh, North Carolina, and brought together a cross section of industry partners, investors, researchers and other select members of the community. The summit featured presenters from around the world, including translational research experts, clinical trial investigators, and the companies poised to take emerging therapies across the finish line. Based on the success of this meeting, the Foundation plans to host this event annually.

▶ *Bradford Manning (left) and Bryan Manning (right) from Two Blind Brothers. Bradford and Bryan were the keynote presenters at the reception dinner.*

▲ *Treasurer Haynes Lea speaking into a microphone participating in the question-and-answer discussion.*

▲ *Co-Founder and Chairman Emeritus Gordon Gund and other summit guests at a table during dinner.*

In the last year, our national network of over 40 volunteer driven chapters came together to raise funds, increase public awareness, and provide support to their Fighting Blindness communities. We are stronger together – as a community.

The ***Fashion & Finance Ball*** on May 22 welcomed 500 attendees to New York City, raising $1.3 million for the Foundation. Visionary Awards were presented to Joseph Papa, chairman of the board and CEO of Bausch Health, Scott Sennett, president and CEO of Tura Inc., and Ryan Simonetti, co-founder and CEO of Convene. The event's emcees were Bradford and Bryan Manning of Two Blind Brothers and the night ended with a special musical performance by Idina Menzel.

▲ *Overhead shot of the entire ballroom filled with guests at the Fashion & Finance Ball.*

◀ *Three event guests who were invited on stage are singing with special guest, Idina Menzel.*

◀ *From left to right: Emily Burch, John Mozeliak, Jason Morris, Michael Lowenbaum on stage while John was presented his award.*

The ***St. Louis Dining in the Dark*** was held on January 18 at the Ritz-Carlton St. Louis and raised over $395,000 for the Foundation. John Mozeliak, St. Louis Cardinals president of baseball operations, was awarded the Foundation's Visionary Service award for his 10 years of support for the Dining in the Dark dinner and the Foundation Fighting Blindness.

▶ *Abbey Fink and Steve Hamby on stage at the Chicago VisionWalk.*

The 14th Annual ***Chicago VisionWalk*** welcomed over 51 teams and 712 participants, with fun like live music and face painting, to celebrate raising more than $280,000 for sight-saving research.

▲ *Team wearing a blue t-shirt that says, "Ask Me! Medical Eye Care" with an image of an eye in the middle, with their arms raised.*

The 13th Annual ***Colorado VisionWalk*** was a beautiful day celebrating the 390 walkers and 35 teams that came out to walk for a cure. The VisionWalkers, volunteers, and various other supporters, raised over $157,800 for research ending blindness.

ASHLYN EXPERIENCES JOY IN DAY-TO-DAY LIFE AFTER SIGHT IS RESTORED

Ashlyn's vision was restored following her treatment with the LUXTURNA gene therapy.

When Ashlyn Reichardt was born, her mother Christina immediately started taking her to eye specialists near their home in Tucson, Arizona, where she was misdiagnosed many times. At the age of 9, full genetic testing finally confirmed that Ashlyn had Leber congenital amaurosis (LCA) with mutations in the RPE65 gene.

◀ *The Reichardt family, from left to right: Christina, Ashlyn, Kailey, and Dan.*

"It took me being Ashlyn's biggest advocate, as an optometrist, knowing that something was going on," says Christina. "I knew we needed full genetic testing and it was much more difficult to order the test many years ago."

Less than a year after receiving the genetic test results, Christina received a phone call from Ashlyn's pediatric ophthalmologist saying Ashlyn was a strong candidate for the recently FDA-approved gene therapy, LUXTURNA®. A few months later, Christina and Ashlyn met with a representative from Spark Therapeutics and in May 2018, Christina received a call to be at the Children's Hospital in Los Angeles for Ashlyn's surgery in just a few days.

So, from the end of May into early June, Ashlyn received the LUXTURNA treatment for both of her eyes. Ashlyn was the

▲ *Ashlyn with her sister, Kailey, helping her put her pajama's on right before her first surgery at the Children's Hospital in Los Angeles.*

first female child in the United States to have the treatment since the FDA approval in December 2017.

Although Ashlyn and her family were scared before the surgery, they all felt hopeful.

"We were excited for the possibility of her vision changing, but we were also scared as one of the first to get the treatment after the FDA approval," says Christina. "We didn't know what the outcome would be, and that unknown made us nervous."

A day after the surgery on Ashlyn's first eye, she already started to notice a difference in her vision, but it wasn't easy. Everything seemed very bright to Ashlyn, which kept her in a dark room, but a few days later she was seeing things and very excited. Then Ashlyn went into surgery again for her other eye.

"The second eye was harder for us all," recalls Christina. "But a few days after the second eye surgery, Ashlyn was seeing things she had never seen before."

▲ *Ashlyn and her sister Kailey riding in a boat looking at the fall foliage on their vacation to Pinetop, Arizona. This was Ashlyn's first-time seeing tree leaves change colors.*

The whole Reichardt family all stayed in Los Angeles for over a month before they could go home. But once they got home, Ashlyn experienced seeing a lot of "firsts."

"The first thing Ashlyn wanted to see was our dogs," says Christina. "She said that they looked a lot different in her head. And then she ran to our fish tank and noticed the fish were all different colors. She was so excited that she could actually see their eyes and tails."

Ashlyn's vision continued to improve, and she experienced joy from just living her typical day-to-day life.

"We were late to school one day because she saw her shadow for the first time and just wanted to watch it," recalls Christina. "The first few weeks home were like having a new baby or toddler, everything was brand new to her."

Now 11 years old, Ashlyn's future has gotten brighter and transformed into one with sight.

"My best piece of advice is to believe in the process," says Christina. "Knowing Ashlyn's genetic mutation was so helpful for us – it was everything to get this treatment and it has changed our lives completely."

2019 RESEARCH INVESTMENTS

Thanks to our successful fiscal year of fundraising, the Foundation has committed $6.5 million for 14 new research projects for inherited retinal diseases. The newly funded research efforts include development of a CRISPR/Cas9 therapy for retinitis pigmentosa, a retinal imaging technique using artificial intelligence, and several therapies that have strong potential to treat a wide range of inherited retinal diseases. The Foundation currently funds a total of 80 research grants. Listed below are highlights of some of the new grants:

Next generation optogenetics for vision restoration ($2.5 million over 5 years)

Optogenetic therapies hold promise for restoring vision in people with advanced retinal disease regardless of their underlying mutation. Deniz Dalkara, PhD, Institut de la Vision (France), and her team are developing an optogenetic therapy that can be administered to different retinal cell types depending on the condition (stage of disease) of the patient's retinal structure. Furthermore, the approach has the potential to provide a higher degree of sensitivity (i.e., better vision) than current optogenetic approaches in clinical trials and translational studies.

Development of small molecule modulator for preserving vision in people with retinitis pigmentosa ($900,000 over 3 years)

Stephen Martin, PhD, and his colleagues at the University of Texas at Austin are developing a small-molecule modulator known as TMEM97/σ2R that can be administered into the vitreous in a slow-release formulation to delay the progression of photoreceptor loss and to preserve vision in people with retinitis pigmentosa. The emerging therapy is designed to work independent of the underlying gene mutation causing the disease. The goal is to develop a drug that can be moved into toxicology studies in preparation for a clinical trial.

Gene editing to treat retinitis pigmentosa ($300,000 over 3 years)

Mutations in the gene rhodopsin (RHO) are a frequent cause of autosomal dominant retinitis pigmentosa. Alberto Auricchio, MD, University "Federico II" (Italy), is developing a gene-editing therapy — homology-independent targeted integration (HITI)

$760 MILLION

$760 million raised for retinal research and public health education since the Foundation Fighting Blindness began in 1971.

36 VisionWalk events throughout the United States, raising over $53 million since 2006 to fund research.

80 GRANTS

Funded in 2019
90+ investigators
67 institutions

35 CLINICAL TRIALS

More than 35 clinical trials underway for potential treatments.

Rare inherited retinal diseases:

200,000+ people affected in the U.S.

4.5 million+ people affected globally

Age-related macular degeneration:

10 million+ people affected in the U.S.

150 million+ people affected globally

More than 270 retinal-disease causing genes identified.

which uses CRISPR/Cas9 — to cut and replace both the normal and mutated copies of RHO. The therapy is being designed to work for all mutations in RHO.

Elucidating the disease pathway of cone dystrophy with supernormal rod response ($300,000 over 3 years)

CDSRR is diagnosed in children and young adults based on a very unusual pattern of electrical activity in the retina. The retinal disease is caused by mutations in the gene KCNV2. Sheila Baker, PhD, University of Iowa, is using CRISPR/Cas9 to create a mouse model of CDSSR so that she and the research community can better understand how the disease occurs and identify targets for treatments.

Identifying optimal CRB1 protein for treating Leber congenital amaurosis and retinitis pigmentosa ($300,000 over 3 years)

Mutations in the gene CRB1 cause Leber congenital amaurosis (LCA) and retinitis pigmentosa (RP). The CRB1 gene can express different forms (isoforms) of protein. Jeremy Kay, PhD, Duke University, believes he has identified a CRB1 isoform that will work well in a gene therapy for people with CRB1-related mutations. He is evaluating the rescue efficacy and expression pattern of this isoform. His efforts will help CRB1 gene therapy developers design the optimal gene therapy for people with CRB1 mutations.

Evaluating subsets of RPE cells affected by age-related macular degeneration ($300,000 over 3 years)

Age-related macular degeneration (AMD) is a complex condition causing degeneration of retinal pigment epithelial (RPE) cells, which provide a support system for photoreceptors. When RPE cells don't work properly or die off, photoreceptors degenerate. James Handa, MD, Johns Hopkins University, believes subsets of RPE cells are pathologic in AMD and play a lead role in driving the disease. His goal is to better understand and identify the changes in diseased RPE cell subsets and investigate potential targets for treating them.

Deep machine learning to identify functional and structural changes in photoreceptors ($300,000 over 3 years)

Yi-Zhong Wang, PhD, Retina Foundation of the Southwest, is using optical coherence tomography (OCT) enhanced with deep machine learning, a form of artificial intelligence, as a highly sensitive approach for measuring functional and structural changes in outer segments, the light-sensitive protrusions in photoreceptors. The project includes "training" the neural network to evaluate features of outer segments. The research team will be evaluating the technique in RUSH2A (USH2A natural history study) participants for correlating outer segment structure with visual field sensitivity. The project may ultimately help researchers identify an endpoint for treatments in clinical trials.

Predicting disease progress in people with Stargardt disease using a deep learning algorithm ($65,000 over 1 year)

Diana Davis Spencer Clinical Fellowship

Peter Zhao, MD, University of Michigan, is developing a deep learning algorithm, a form of artificial intelligence, to analyze fundus (back of the eye) autofluorescence images of patients with Stargardt disease. Developing a deep learning algorithm will facilitate the identification of patients whose disease is more likely to progress in the near future. Patients at risk of disease progression make good candidates for clinical trials of treatments that are designed to slow or halt vision loss.

Investigating retinal structure and function in people with X-linked retinitis pigmentosa caused by RPGR mutations ($65,000 over 1 year)

Diana Davis Spencer Clinical Fellowship

Marco Nassisi, MD, Institut de la Vision (France), is conducting a retrospective analysis of collected data, including functional and structural parameters to model disease progression, in people with X-linked retinitis pigmentosa caused by RPGR mutations. His goal is to better understand how different mutations in the gene affect vision loss. Ultimately, he wants to determine endpoints that can be used in clinical trials of gene therapies and other emerging treatments.

GROWTH IN MY RETINA TRACKER® REGISTRY

One of the key requirements for moving promising clinical programs forward is the ability to find the right patients to enroll in the clinical trials. My Retina Tracker Registry is a research database for inherited retinal diseases managed by the Foundation. We are encouraged by the large number of patients enrolling in the Registry who are helping to drive progress in the field, including access to clinical trials.

My Retina Tracker Expansion for No-Cost Genetic Testing

We are pleased to build on the benefits of My Retina Tracker Registry through an expanded open-access program offering patients with inherited retinal diseases (IRDs) no-cost genetic testing and genetic counseling. The new program, My Retina Tracker Program, in partnership with Blueprint Genetics and InformedDNA, is an expansion of the genetic testing program established in 2017. Previously, patients were only eligible for the testing if they were seen by an eligible specialist and if they enrolled in the Registry.

My Retina Tracker Program will now be available to all U.S. patients with an IRD diagnosis. The registry currently includes more than 24,000 participants, with over 13,000 detailed profiles and almost 7,000 individuals having been genetically tested. The Foundation's goal over the next five years is to have 40,000 registry members, with more than 20,000 with genotype information available.

MY RETINA TRACKER PROGRAM, MY STORY

Susan, who was diagnosed at age 28 with retinitis pigmentosa (RP), was experiencing relatively rapid vision loss. Six months later, Susan had full renal failure from polycystic kidney disease and had an immediate kidney transplant. She has no family history of RP but her two younger brothers had both died young of kidney disease.

In search to learn more about her eye disease, Susan received genetic testing for her RP through a genetic testing program that focused on analyzing only 32 genes – mainly involved in early onset RP. One pathogenic RPE65 variant was identified. Her local retinal specialist said she probably had RPE65-disease and would qualify for a new vision-restoring gene therapy called LUXTURNA. However, she was told that she did not qualify. RP caused by RPE65 mutations is a recessively inherited disease and she would need to have two mutations in her genes identified to qualify.

Susan had not received genetic counseling with the first genetic test and was then referred to a genetic counselor. This counselor informed Susan that her retinal disease was not typical of RPE65 disease and was probably due to a different genetic cause. The counselor helped her connect with a different retina specialist who worked with her to find and enroll in the My Retina Tracker Program testing program. My Retina Tracker Program provides

a comprehensive test of 266 retinal disease genes. This time, her test came back with a clear diagnosis – two mutated copies of the NPHP1 gene, which is the cause of her RP – and is also known to cause kidney disease.

About her experience, Susan said, "I was first told that genetic testing wasn't important for me. I found another doctor and found the Foundation's program. And now look, this genetic testing has answered questions for me and my family that I never would have had otherwise. I started out frustrated that I didn't qualify for gene therapy, but now I'm so grateful for everything I've learned about my disease. This has given me hope."

▶ *Susan showing her muscles with her t-shirt that says, "Retinitis Pigmentosa Warrior. It's not for the weak."*

CLINICAL TRIAL PIPELINE

GENE THERAPIES	Progress
Achromatopsia (CNGB3) – AGTC	Phase 1/2
Achromatopsia (CNGB3) – MeiraGTx	Phase 1/2
Achromatopsia (CNGA3) – AGTC	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD (Dry) – Gyroscope	Phase 1/2
Choroideremia (REP1) – Nightstar	Phase 3
Choroideremia (REP1) – Spark	Phase 1/2
Choroideremia (REP1) – Tubingen Hosp	Phase 2
LCA and RP (RPE65) – MeiraGTx	Phase 1/2
LCA and RP (RPE65) – Spark	FDA Approved
RP (PDE6B) – Horama	Phase 1/2
RP, Usher, others (optogenetic) – Allergan	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP (RLBP1) – Novartis	Phase 1/2
Retinoschisis (RS1) – AGTC	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
Stargardt disease (ABCA4) – Sanofi	Phase 1/2
Usher syndrome 1B (MYO7A) – Sanofi	Phase 1/2
X-linked RP (RPGR) – AGTC	Phase 1/2
X-linked RP (RPGR) – MeiraGTx	Phase 1/2
X-linked RP (RPGR) – Nightstar	Phase 3 Pen.

CELL-BASED THERAPIES	Progress
AMD-dry (RPE) – Astellas	Phase 1/2
AMD-dry (RPE) – Cell Cure	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

MOLECULES, PROTEINS, AONS	Progress
AMD-dry (C3 inhibitor) – Apellis	Phase 3
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 vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Iveric bio	Phase 2
Stargardt disease (anti-RBP4) – Belite Bio	Phase 1
Usher syndrome 2A (AON) – ProQR	Phase 1/2

A MESSAGE FROM OUR TREASURER

As the leading nonprofit funder of inherited retinal disease research, fiscal responsibility is at the core of our organization. The 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.

I am pleased to provide you the statement of activities and financial position for the Foundation Fighting Blindness' fiscal year ending June 30, 2019. For the year, we outperformed our planned budget with revenue and support of $56 million, which includes $24 million restricted for the recently established Retinal Degeneration Fund (RD Fund). For Fiscal 2019 we deployed $23 million towards research, public health and education expense of $1.7 million and operating expense of $9.1 million.

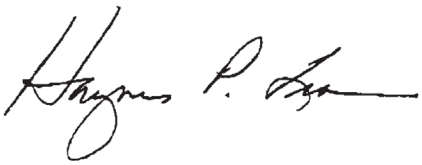
Compared to prior years, this level of support is significant, and allowed us to launch the RD Fund, which now has more than $70 million in initial funding. The RD Fund invests in companies with projects that can be in clinical testing in less than 18 to 24 months. As we work to effectively deploy these funds over the next several years, we will add to our near-term revenue by generating investment income on the funds, while also furthering our research mission. Other Revenue, including investment income, totaled approximately $7 million in Fiscal Year 2019.

As of June 30, 2019, we had Net Assets of approximately $153 million, which included $112 million that is committed to scientific research through donor restricted funding for grants, endowments and the RD Fund. As we embark on our new five-year strategic plan, our ability to continue raising funds is critical. We have allocated $105 million over this period for scientific research.

In summary, while there have been major advances in treatments for patients with blinding retinal degenerative

diseases, there remains tremendous need to expand scientific research and find new therapies. No matter what the diagnosis, our collective drive can enable each individual to thrive. With everyone from our community working together, we are stronger and can accomplish our mission.

With sincere gratitude to all of our donors, volunteers, staff and researchers,



Haynes P. Lea, Treasurer

STATEMENT OF ACTIVITIES

REVENUE AND SUPPORT	June 30, 2019	June 30, 2018
Contributions	$37,179,000	$7,795,000
Special events, net of direct	$7,035,000	$6,410,000
Bequests	$5,185,000	$7,741,000
Other revenue	$6,629,000	$1,206,000
TOTAL REVENUE	**$56,028,000**	**$23,152,000**

EXPENSES	June 30, 2019	June 30, 2018
Research	$23,049,000	$20,550,000
Public Health Education	$1,689,000	$2,324,000
Management	$2,079,000	$2,565,000
Fundraising	$7,080,000	$6,563,000
TOTAL EXPENSES	**$33,897,000**	**$32,002,000**

TOTAL CHANGE IN NET ASSETS	$22,131,000	$(8,850,000)

TARGET SPENDING ALLOCATIONS

71%
RESEARCH, INCLUDING GRANTS AND INVESTMENTS

17%
FUNDRAISING

6%
PUBLIC HEALTH EDUCATION

6%
ADMINISTRATION

STATEMENT OF FINANCIAL POSITION

ASSETS	June 30, 2019	June 30, 2018
Cash and investments	$132,958,000	$101,556,000
Pledges receivable, net	$26,255,000	$29,203,000
Other assets	$1,787,000	$1,878,000
Trusts and other funds	$7,450,000	$7,924,000
Fixed assets, net	$1,329,000	$1,385,000
TOTAL ASSETS	**$169,779,000**	**$141,946,000**

LIABILITIES	June 30, 2019	June 30, 2018
Accounts payable and accrued liabilities	$1,589,000	$1,853,500
Research grants payable	$13,981,000	$6,842,000
Deferred revenues	$316,000	$904,500
Liabilities under trusts and other funds	$929,000	$1,513,000
TOTAL LIABILITIES	**$16,815,000**	**$11,113,000**

NET ASSETS	**$152,964,000**	**$130,833,000**
TOTAL LIABILITIES AND NET ASSETS	**$169,779,000**	**$141,946,000**

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The Foundation Fighting Blindness maintains a network of chapters across the country which hold seminars and meetings that provide information on research, low vision resources, and other helpful industry topics that provide support to these communities.

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