2023 Annual Report

FOUNDATION FIGHTING BLINDNESS

The Foundation Fighting Blindness is the global leader driving the research for blinding retinal diseases such as retinitis pigmentosa, Usher syndrome, Stargardt disease, Leber congenital amaurosis, age-related macular degeneration, and more.

The Foundation has made remarkable strides in advancing treatments and cures for these diseases in the last year, continuing to drive innovative science and filling the pipeline with clinical trials. This impact on the retinal field would not be possible without the generosity of our steadfast donors. But our work is not complete, and the Foundation will continue to work tirelessly toward our urgent mission.

Learn more and stay connected with the Foundation by calling **(800) 683-5555** or visiting: **FightingBlindness.org**

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A Message from Our Board Chair

As board chair of the Foundation Fighting Blindness, I want to thank you, our partners in the quest to end blinding retinal diseases, for your ongoing support. This Annual Report is full of success stories—in research, fundraising, and fiscal management—all made possible because of the support of each of you.

For me, this report is more than a look back at a fiscal year. I am now in my final year as chair, and next year's letter will come from the prodigious Karen Petrou, who will take my place as chair in July 2024. So today, I am looking back at both a successful year and at my tenure as chair, which started in 2016. I succeeded Foundation co-founder Gordon Gund, and my time in this position has been guided by one simple goal—**making sure Gordon's legacy is continued and preserved so that all people with retinal diseases have treatments and cures.**

Since 2016, the organization has brought on some tremendous staff leadership, notably hiring Ben Yerxa, followed by Jason Menzo, our current CEO. With the changes in volunteer leadership—from Gordon to me and soon to Karen—the organization has proved itself facile at transition. We've built a staff and board that is strong, talented, and deep in layers, that understands the difficulties of this work and the benefits of great performance.

We continued to make certain that the organization will remain strong for the future by creating new ways for young leadership to engage, like our Strategic Council, a group of young professionals with an affinity for the Foundation Fighting Blindness mission who represent the next generation of Foundation leaders.

We continue to grow My Retina Tracker® Registry, which now has more than 25,000 members in the database so that we know where people who are affected are and can get them involved in clinical studies. We've expanded the Chapter network, a major pipeline of volunteer support, through "Lulie's Next Chapter." And science education has become a hallmark of the organization, with popular podcasts and seminars.

The Foundation has continued to fund innovative and cutting-edge research in promising areas such as genetics, gene and stem-cell therapies, retinal cell transplantation, and pharmaceutical and nutritional therapies. Today, there are more than 45 clinical research programs in our field, and the Foundation has played a substantial role in the great majority of these, where 15 years ago, we had four or five. We've successfully helped to fill the needs in the scientific community by creating a clinical research network that stretches around the world and setting a protocol for exams for retinal patients, which never existed before. Along the way, we sought to think even bigger and created the Retinal Degeneration Fund (RD Fund). Since its inception, it has resulted in leveraging more than $500 million of non-Foundation funds into the field. As you can see, we provide a critical bridge between discovery research, proof of concept, and corporate investment in clinical trials and commercialization. All of this is good news in the quest for cures.

Still, there's far more good news than will fit in this letter, but I can say that the successes of the last eight years are the result of your financial support. It takes you, our donors, to propel a top-notch staff and board working as a team alongside the expertise of countless scientists and clinicians.

I can say that today, there is a stream of successful, FDA-approved interventions, with more on the horizon. Best of all, I can say confidently that Gordon Gund's legacy is stronger than ever.

With gratitude,

David Brint
Chair
Board of Directors

Image Description: Headshot of Board Chair, David Brint.

A Message from Our CEO

As I begin my second year as CEO here at the Foundation, I want to thank you for your commitment to the Foundation and your continued support. I have been privileged to serve with board chair David Brint since 2018, first as COO, then as President, and now as CEO. David is a long-time advocate and supporter of the Foundation, and his leadership and vision have moved us closer than ever to achieving our mission. I hope you'll join me in congratulating him on his brilliant tenure in the position, as this will be his last year as board chair.

I believe this is a significant moment of change, so I'm taking this opportunity to both look back and into the future.

This report demonstrates a strong financial year, thanks to supporters like you. We raised tens of millions of dollars to drive the research to provide treatments and cures, more than 80 percent of which went directly to mission-related activities.

Last year, we had ten scientific programs advance to the next stage of clinical development, meaning these programs moved into human testing for the first time or progressed to more advanced stages of clinical testing with the aim of eventual FDA approval. We've grown the My Retina Tracker Registry to more than 25,000 members, we grew the number of donors supporting the organization to over 31,500, and we're strengthening our Chapters like never before.

There have been so many significant events in the past year, and where we stand today serves as the foundational cornerstone for our ongoing journey.

We don't take lightly the trust you place in us with your precious donations to sustain the flow of life-changing contributions that are essential to the science pipeline. We expect more treatments to move to the next stage of development and simultaneously expect to continue to make groundbreaking investments in companies in our space through the RD Fund.

In the year ahead, we commit to engaging our community deeper and more significantly than ever before and driving the field faster and further with a singular focus: to bring forth more treatments and cures.

As we plan for tomorrow, we are engaging the global scientific community to identify the current gaps in the field, and designing programs to best fill those gaps. The success of the ongoing *Victory for Vision* campaign is an example of what we can do when we join together to win this battle. We did it with you, and I believe it's the kind of success we will continue to see as Foundation Fighting Blindness moves into this next chapter.

In closing, when I think about the Foundation of tomorrow—years ahead, not just the next year—it's very simple. I want us to be recognized as the undisputed global leader advancing cures for blinding diseases.

Thanks for being an important part of making that a reality.

Sincerely,

Jason Menzo
Chief Executive Officer

Image Description: Headshot of CEO, Jason Menzo.

Impressive Research Progress for 2023

Nearly 50 clinical trials were underway for emerging therapies for retinal degenerative diseases by the end of the Foundation's Fiscal Year 2023 (FY23). Excitingly, the U.S. Food & Drug Administration (FDA) also approved the first-ever drugs for geographic atrophy (GA) secondary to advanced dry age-related macular degeneration (AMD) over the past year, and the Foundation has partnered with the companies bringing these therapeutics to patients. Research highlights for FY23 also include progress in clinical trials for gene-agnostic therapies for preserving vision and restoring vision for people with advanced vision loss.

FDA Approved Apellis' SYFOVRE™ for Treatment of Geographic Atrophy Secondary to Advanced Dry AMD

Apellis Pharmaceuticals received approval from the FDA for SYFOVRE™ (pegcetacoplan injection), a drug for people with geographic atrophy (GA) secondary to age-related macular degeneration (AMD). The first-ever drug approved for GA, SYFOVRE, slowed the growth of lesions (areas of retinal cell loss) in two Phase 3 clinical trials. SYFOVRE is designed to slow the progression of GA by inhibiting C3, a protein in the complement system. While complement is part of the human immune system that wards off harmful bacteria and viruses, it can cause damage if not controlled properly.

Atsena's LCA-GUCY2D Gene Therapy Improves Vision in Phase 1/2 Clinical Trial

Atsena Therapeutics announced positive results from its Phase 1/2 gene therapy clinical trial for people with Leber congenital amaurosis type 1 (LCA1), which is caused by mutations in the gene GUCY2D. Overall, the gene therapy, ATSN-101, was well tolerated. The nine patients receiving the highest dose of ATSN-101 had clinically meaningful vision improvements as measured by a full-field stimulus test (FST), which measures the patient's ability to respond to different levels of light, and by their ability to navigate a multi-luminance mobility course. Atsena has received an investment from the Foundation's RD Fund, a venture philanthropy fund for emerging treatments in, or moving toward, early-stage clinical trials.

SparingVision Initiated Clinical Trial of Cone-Preserving Gene Therapy for RP

SparingVision launched a Phase 1/2 clinical trial for its gene-independent, cone-preserving therapy SPVN06 for people with retinitis pigmentosa (RP). The trial is underway at the University of Pittsburgh Medical Center and Centre Hospitalier National d'Ophtalmologie des Quinze-Vingts (CHNO XV-XX), Paris. The Foundation's RD Fund is one of the investors in SparingVision. The Foundation also provided several years of research grant funding for the preclinical development of the treatment.

Belite Bio Launched Phase 3 Clinical Trial for Stargardt Disease Drug

Belite Bio began dosing adolescent patients (ages 12-18) with Stargardt disease in DRAGON, its Phase 3 clinical trial evaluating Tinlarebant, an emerging oral medication designed to slow disease progression and vision loss. The company initiated the Phase 3 clinical trial in the US, UK, Germany, Belgium, Switzerland, Hong Kong, Taiwan, and Australia.

Approximately 60 patients are targeted for enrollment in the placebo-controlled study. Tinlarebant is designed to inhibit a protein known as retinol binding protein 4 (RBP4) to reduce the uptake of vitamin A to the retina, thereby decreasing the production and accumulation of toxic vitamin A byproducts, which are the hallmark of Stargardt disease.

Bionic Sight Reported Meaningful Vision Improvements for RP Patients Receiving Highest Dose of its Emerging Optogenetic Therapy

All 12 patients with advanced vision loss from retinitis pigmentosa (RP) dosed in a Phase 1/2 clinical trial for Bionic Sight's emerging optogenetic treatment demonstrated significant vision improvements. Those receiving the highest dose of the therapy had the most vision restored. The four top responders in the trial gained the ability to recognize shapes and objects. Bionic Sight's approach involves two components: 1) A one-time optogenetic treatment that enables expression of a light sensitive protein in retinal ganglion cells which survive after photoreceptors are lost to an advanced retinal disease like RP. 2) A device, worn like a pair of glasses, that captures the scene a person is looking at and generates vision-enabling code, which is sent to the light-sensitive ganglion cells, and then on to the brain.

Vision Improved for RP Patients in Phase 2B Clinical Trial of Nanoscope's Optogenetic Therapy

Nanoscope Therapeutics reported that 16 of 18 patients with advanced vision loss from retinitis pigmentosa in the RESTORE Phase 2b clinical trial for its optogenetic therapy were able to better complete at least one of two tests under low luminance: a simple Y mobility test or a shape recognition test. The treatment, a small drop of liquid delivered by an intravitreal injection, uses a human-engineered virus to deliver copies of a Multi-Characteristic Opsin (MCO) gene to bipolar cells — cells that don't normally sense light but often survive after photoreceptors are lost to advanced retinal disease. The MCO enables bipolar cells to respond to light.

A Message from Our Treasurer

On behalf of the board and staff of the Foundation, I want to thank you for your ongoing support. Having completed my third year in this position, I am more confident than ever that the level of care with which the Foundation treats every donated dollar is truly remarkable—all with the goal of continuing to fund and expand research that will produce treatments and cures for blinding retinal diseases.

I am honored to introduce to you the statement of activities and financial position for the fiscal year ending June 30, 2023. Total revenue came in at $26.0M this fiscal year. We deployed $32.7M towards research, spent $2.8M on public health and education, and incurred fundraising and management expenses of $9.9M.

In nearly every category, the Foundation finished well above plan for the year:

- In terms of net major gifts, we exceeded the plan by $2.5M due to you, our donors, and the continued effect of success we've seen due to the Victory for Vision campaign.
- Organizational partnerships, which are among the newer areas of focus for the Foundation from a fundraising perspective, continue to outperform expectations and were $200,000 higher than budget.
- Operating expenses were $1.0M below plan as the Foundation continued to tightly manage spending.

Events revenue, which continues to recover from the impact of the pandemic years, was $800,000 higher than last year. While short of budget, the team is always looking for new ways to reinvigorate events and ensure they are meaningful to you and all those who participate.

Our legacy giving, which is the most unpredictable of our fundraising buckets, still continues to welcome new members into the Reintsma 2025 Legacy Society. I encourage everyone to make a gift to the Foundation as part of their legacy plan.

Despite an ever-changing fundraising landscape that has been full of challenges for the last several years, the organization continues to outperform and is proactively shifting to meet the challenges of the new landscape. We're continuing to drive fundraising in the areas in which we've always focused but also expanding into organizational partnerships, federal funding, social media and other development efforts.

As the parent of a child who is impacted by a blinding retinal disease, I am confident that the field is moving forward and that more treatments and cures will be available. I am sure that, with your support, the Foundation Fighting Blindness will play a crucial role in them.

Gratefully,

Jason Morris
Treasurer

Financial Summary

Statement of Activities

	June 30, 2023	June 30, 2022
Revenue and Support		
Contributions	$19,665,000	$31,789,000
Special Events Revenue, Net of Direct Expense	$6,282,000	$5,483,000
Bequests	$1,573,000	$313,000
Other Revenue	($1,534,000)	($2,308,000)
Total Revenue	$25,986,000	$35,277,000
Expenses		
Research	$32,721,000	$27,388,000
Public Health Education	$2,782,000	$2,699,000
Management	$2,912,000	$3,075,000
Fundraising	$7,027,000	$7,079,000
Total Expenses	$45,442,000	$40,241,000
Total Change in Net Assets	($19,456,000)	($4,964,000)
Issuance of Common and Preferred Stock by Opus	$4,500,000	$4,459,000
Deconsolidation of Opus	($2,174,000)	($0)
Net Assets as of June 30	$147,458,000	$164,589,000

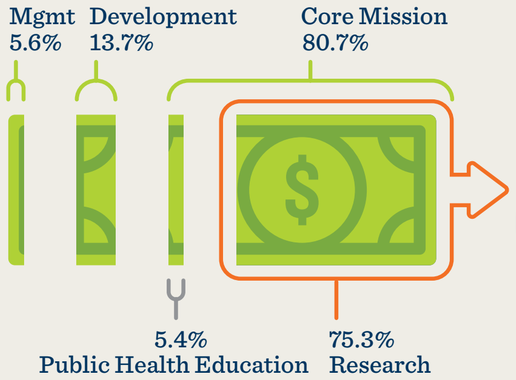
Statement of Financial Position

	June 30, 2023	June 30, 2022
Assets		
Cash and Investements	$101,704,000	$112,923,000
RD Fund Investments	$37,780,000	$37,104,000
Pledges Receivable, Net	$19,464,000	$23,017,000
Other Assets	$3,325,000	$1,275,000
Trust and Other Funds	$8,012,000	$7,597,000
Fixed Assets, Net	$671,000	$813,000
Total Assets	$170,956,000	$182,729,000
Liabilities		
Accounts Payable and Accrued Liabilities	$2,060,000	$2,108,000
Research Grants Payable	$20,414,000	$15,215,000
Deferred Revenues	$443,000	$210,000
Liabilities Under Trust and Other Funds	$581,000	$607,000
Total Liabilities	$23,498,000	$18,140,000
Net Assets	$147,458,000	$164,589,000
Total Liabilities and Net Assets	$170,956,000	$182,729,000

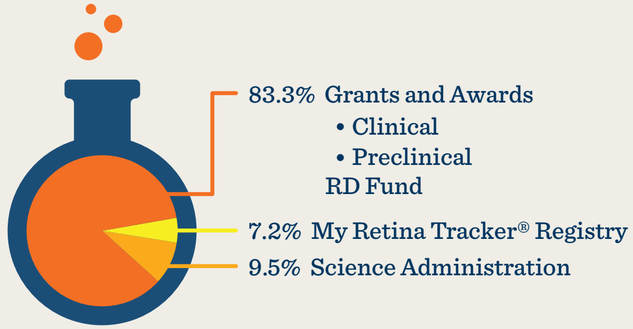
Note: For additional information on the Foundation's financial results, please refer to the FY23 audited statements available on the Foundation website.

Where Does the Money Go?

DONATION ALLOCATIONS



RESEARCH BREAKDOWN



A Message from Our Scientific Advisory Board Chair

It is my honor to provide this Annual Report message on behalf of the Scientific Advisory Board. Since the Foundation routinely receives far more requests for funding than we can fulfill, our job is to curate the best and most promising science. Each one of us loves what we do, and we are all dedicated to the mission.

For perspective, in the last fiscal year, we received **137 letters of intent and requested 57 applications.** It's both an exciting and daunting task, but one that is crucial in order for the Foundation to make the best use of its funds and stay laser-focused on its mission to provide treatments and cures for blinding retinal diseases.

It bears repeating that the Foundation Fighting Blindness is the leading non-governmental source of funding for research on retinal diseases in the world. It has provided inspiration and means for the brightest scientists in the field to apply their talents to the study of these understudied, rare diseases so that people are motivated to try to apply their research efforts to the development of treatments and cures.

The strength of the Scientific Advisory Board is part of what distinguishes the Foundation Fighting Blindness. And in addition to the ongoing work of assessing the most promising funding requests, we are currently soliciting feedback from all the members of this board to identify gaps in the field and progress that has been made, and we're using this information to inform the strategic planning process.

Thanks to your generous support, the Foundation is creating infrastructure so that we can better understand disease mechanisms and identify novel therapies. Thanks to the Foundation and its Clinical Consortium, we can do natural history studies of a rare inherited disease to develop outcome measures that can then be used to support clinical trials. Those trials will enable us to assess new treatments that are being discovered by the basic scientists.

That pipeline—from the basic mechanisms of disease, all the way to clinical trials—is truly remarkable. Your support is a critical piece of the pipeline. Because with so many basic science requests going unfunded—and plenty of treatments and cures left to discover—the Foundation's need for funding is more urgent than ever before.

To all of you who help make our work possible, thank you.

Sincerely,

Jacque Duncan

Jacque Duncan, MD
Chair, Scientific Advisory Board
Professor and Chair
Department of Ophthalmology
Wayne and Gladys Valley Center for Vision
University of California, San Francisco

To view a full list of Scientific Advisory Board members, visit:
www.FightingBlindness.org/about/scientific-advisory-board

Foundation Funds 25 New Grants Totaling $15.1 Million in FY23

The Foundation Fighting Blindness added 25 new research projects to its portfolio, an investment totaling $15.1 million, during its fiscal year 2023 (ending June 30, 2023). Project awards ranged from early-stage lab research to identify treatment targets to translational efforts for advancing emerging therapies toward clinical trials.

"Excitingly, 15 of the new awardees for FY23 are researchers never previously funded by the Foundation," said Claire Gelfman, PhD, chief scientific officer. "We make the greatest impact in driving our urgent mission when we continually infuse our efforts with new ideas and research talent."

Research grants were selected after a rigorous review process conducted by the Foundation's Scientific Advisory Board, which is comprised of more than 60 of the world's leading retinal scientists and clinicians.

The Foundation's current research portfolio funds a total of 93 grants. The research projects are conducted by more than 96 research investigators at 71 institutions around the world. In addition to funding researchers in the United States, the Foundation funding extends internationally to laboratories in Australia, Belgium, Brazil, Canada, Denmark, England, Finland, France, Germany, Israel, Italy, Mexico, the Netherlands, Poland, Spain, and Switzerland.

Image Description: A male scientist looking into a microscope.

Types of Awards

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

The **Free Family Age-Related Macular Degeneration (AMD) Award** funding strives to find solutions for early, dry AMD.

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

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

Clinical Innovation Awards advance development of endpoints in IRD clinical trials that might be accepted by regulatory agencies for improving IRD patients' conditions or slowing disease progression.

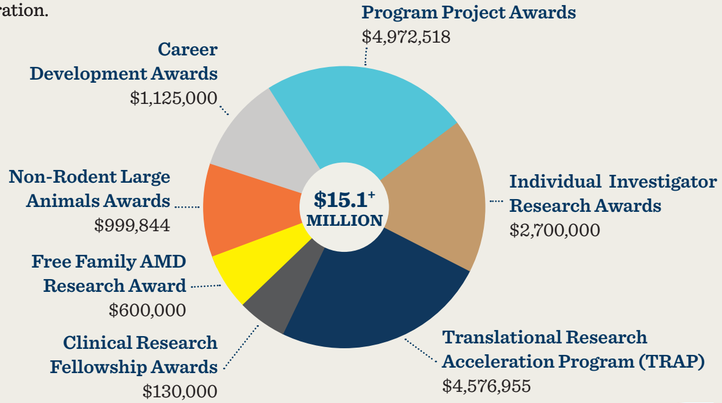
The **Career Development Awards** support physicians' and physician-scientists' career development through a multi-level, tiered approach across the duration of their careers. Each level represents a new "stage" in the career progression of a physician or physician-scientist, at a point when they can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

Clinical Research Fellowship Awards provide funding for medical doctors in clinical fellowships examining orphan inherited retinal degenerations. The goal is to increase the number of clinician-scientists with expertise and a commitment to providing clinical care to patients with inherited retinal disease. This program prepares doctors for careers in academic medicine and provides critical training in an environment that fosters research to develop preventions, treatments, and cures for IRDs.

The **Research Core Award** provides funding to individuals or teams striving to identify, develop, characterize, and support relevant large animal models of IRD or dry AMD that are poorly modeled in rodents and for which canine models do not currently exist.

New Awards

Fiscal Year 2023



New Grants

Translational Research Acceleration Program Awards (TRAP)

Krzysztof Palczewski, PhD – $900,000
The Regents of the University of California, Irvine
"Correcting previously untreatable retinal degenerative diseases using twin prime editing."

François Paquet-Durand, PhD – $989,000
Mireca Medicines GmbH
"Clinical translation of a mutation-independent treatment for hereditary retinal degeneration using BlockPKG, an inhibitory cGMP analogue."

Kathryn Pepple, MD, PhD – $900,000
University of Washington
"Evaluating mitigation strategies for intravitreal viral vector-mediated inflammation across animal models."

Baerbel Rohrer, PhD – $900,000
MitoChem Therapeutics
"Targeting the molecule FUS for neuroprotection: A novel therapeutic approach in retinal degeneration."

Renee Ryals, PhD – $887,955
Casey Eye Institute, Oregon Health & Science University
"Lipid nanoparticle-mediated gene editing for IRD patients harboring PRPH2 mutations."

Free Family AMD Research Award

Stephen Tsang, MD, PhD, and Marta Olah, PhD – $600,000
Columbia University
"ARMS2/HTRA1 in non–cell-autonomous oxidative and anti-inflammatory therapeutic targeting."

Program Project Awards

Silvia Finnemann, PhD – $2,472,518
Fordham University
"A novel, rationally designed pharmacological approach to countering vision loss in a preclinical model of MERTK-associated retinitis pigmentosa."

Dror Sharon, PhD – $2,500,000
Hadassah-Hebrew University Medical Center
"In vivo retinal RNA editing using the cellular adenosine deaminase acting on RNA (ADAR) enzyme."

Individual Investigator Research Awards

Esther Biswas-Fiss, PhD – $300,000
University of Delaware
"Deciphering the impact of ABCA4 genetic variants of unknown significance in inherited retinal disease prognosis."

Yu Holly Chen, PhD – $300,000
University of Alabama at Birmingham
"Restoring extracellular matrix signaling between Müller glia and photoreceptors for therapies of inherited retinal degeneration."

Frauke Coppieters, MSc, PhD – $300,000
Ghent University
"Long non-coding RNAs (lncRNAs) as molecular drivers and therapeutics targets of inherited retinal disease."

Manuel Irimia, PhD – $300,000
Centre for Genomic Regulation (CRG)
"Identification, validation, and modulation of uncharacterized splicing mutations in inherited retinal diseases."

Simon Petersen-Jones, DVetMed, PhD, DVOphthal, DECVO – $300,000
Michigan State University
"Knock-down and replacement therapy for dominant CRX-associated retinopathies."

Peter Quinn, PhD – $300,000
Columbia University
"Prime editing for PRPH2 inherited retinal dystrophies."

Thomas Reh, PhD – $300,000
University of Washington
"Reprogramming human Muller glia to retinal progenitors and neurons."

Melanie Samuel, PhD – $300,000
Baylor College of Medicine
"Targeting microglia to prevent retinal neuron loss in inherited retinal degenerations."

Career Development Awards

Robert Hyde, MD, PhD - $375,000
University of Illinois-Chicago
"Inner retinal dysfunction in retinitis pigmentosa."

Debarshi Mustafi, MD, PhD – $375,000
University of Washington
"Deciphering the missing heritability in inherited retinal diseases with targeted long-read genome sequencing."

Katherine Uyhazi, MD, PhD – $375,000
University of Pennsylvania
"Investigating the heterogeneity of photoreceptor precursor cells for retinal regeneration."

Learn more by visiting:
www.FightingBlindness.org/funded-grants-2023

Clinical Innovation Award

Ramiro Maldonado, MD – $300,000
Duke University
"Ultracompact hand-held swept-source optical coherence tomography as a novel diagnostic modality for early-onset retinal dystrophies."

Clinical Research Fellowship Awards

Anfisa Ayalon, MD – $65,000
University of Pittsburgh
"Spectral properties of ERG oscillatory potentials in hereditary retinal dystrophies prior to and following the application of gene therapy employing a novel gel-based AAV vector delivery system."

Thales Guimaraes, MD – $65,000
Moorfields Eye Hospital NHS Foundation Trust
"Exploring retinal structure and function in patients with CDH23-associated Usher syndrome."

Research Core: Non-Rodent Large Animal Awards

Martha Neuringer, PhD – $499,844
Oregon Health and Science University
"Creation of a translational nonhuman primate model of Usher Syndrome 1B."

Simon Petersen-Jones, DVetMed, PhD, DVOphthal, DECVO – $500,000
Michigan State University
"Characterization of a large animal Stargardt disease model – suitability for translational therapy trials."

Foundation Supported Workshop

Cold Spring Harbor Laboratory – $5,000
"2023 Cold Spring Harbor Lab – Vision: A Platform for Linking Circuits, Behavior & Perception."

Snapshot of Our Grants and Awards

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

July 2022 – June 2023 (Fiscal Year 2023)
In FY2023, new research awards added to our existing research portfolio:

$15,109,317

137
Letters of intent (LOI) reviewed across all funding opportunities

54
Applications reviewed across all funding opportunities

4
Foundation-sponsored or co-sponsored meetings and workshops

2
Study sections conducted

FY23 Funding Opportunities

- **Clinical Research Fellowship Award** (4 Apps)
- **Career Development Award** (6 Apps)
- **Free Family Initiative in AMD** (14 LOIs | 3 Apps)
- **Program Project Award** (6 LOIs | 3 Apps)
- **Translational Research Acceleration Award** (53 LOIs | 16 Apps)
- **Non-Rodent Large Animal Award** Research Core (8 LOIs | 5 Apps)
- **Individual Investigator Research Award** (56 LOIs | 17 Apps)
  - **Clinical Innovation Award** (folded into Individual Investigator Research Competition)

New Awards

- **2 Clinical Research Fellowship Awards** ($130,000)
- **3 Career Development Awards** ($1,125,000)
- **1 Free Family AMD Award** ($600,000)
- **2 Program Project Awards** ($4,972,518)
- **5 Translational Research Acceleration Awards** ($4,576,955)
- **2 Resource Core Awards** ($999,844)
- **9 Individual Investigator Research Awards** ($2,700,000)
- **1 Supported Workshop** ($5,000)

The awards included in this report are those approved for funding during FY23.

The start date of these awards varies and could be either FY23 or FY24.

Clinical Trial Pipeline

Select Inherited Retinal Disease and Dry AMD Clinical Trials: 46 Trials

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

Gene Therapies (Gene Target)	Progress
Achromatopsia (CNGB3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – MeiraGTx/Janssen	Phase 1/2
Achromatopsia (CNGA3) – Tubingen Hosp	Phase 1/2
AMD-dry, GA (CD59) – Janssen	Phase 2
AMD-dry, GA (RORA) – Ocugen	Phase 1/2
AMD-dry, GA (CFH) – Perceive Bio	Phase 1/2
Batten disease (CLN5) – Neurogene	Phase 1/2
Choroideremia (REP1) – 4DMT	Phase 1/2
LCA (GUCY2D) – Atsena	Phase 1/2
LCA and RP (RPE65) – MeiraGTx/Janssen	Phase 1/2
LCA (LCA5, lebercillin) – Opus Genetics	Phase 1/2
RP (PDE6B) – Coave	Phase 1/2
RP (RLBP1) – Novartis	Phase 1/2
RP & LCA (NR2E3) – Ocugen	Phase 1/2
RP (RdCVF) – SparingVision	Phase 1/2
RP (PDE6A) – Tubingen Hosp	Phase 1/2
Retinoschisis (RS1) – Atsena	Phase 1/2
Retinoschisis (RS1) – NEI	Phase 1/2
Stargardt disease (RORA) – Ocugen	Phase 1/2
X-linked RP (RPGR) – Beacon	Phase 2
X-linked RP (RPGR) – MeiraGTx/Janssen	Phase 3
X-linked RP (RPGR) – 4DMT	Phase 1/2

Small Molecules (Mechanism)	Progress
AMD-dry, GA (deuterated vit. A) – Alkeus	Phase 3
AMD-dry, GA (RBP4 inhibitor) – Belite Bio	Phase 3
RP (NAC-anti-oxidant) – Johns Hopkins	Phase 3
RP (methotrexate) – Aldeyra	Phase 2
RP (small molecule) – Endogena	Phase 1/2
RP (small molecule, photoswitch) – Kiora	Phase 1/2
Stargardt disease (deuterated vit A) – Alkeus	Phase 2
Stargardt disease (C5 inhibitor) – Astellas	Phase 2
Stargardt disease (anti-RBP4) – Belite Bio	Phase 3
Stargardt disease (metformin) – NEI	Phase 1/2
Usher syndrome (NACA-anti-oxidant) – Nacuity	Phase 2

RNA/Other Therapies (Mechanism)	Progress
AMD-dry, GA (CB inhibitor) – Ionis	Phase 2
RP-PRPF31 (CNOT3) – PYC	Phase 1/2
RP, Usher, others (optogenetic) – Bionic Sight	Phase 1/2
RP, Usher, others (optogenetic) – GenSight	Phase 1/2
RP, Usher, others (optogenetic) – Nanoscope	Phase 2
Stargardt disease (optogenetic) – Nanoscope	Phase 2

Cell - Based Therapies (Cell Type)	Progress
AMD-dry, GA (RPE) – Astellas	Phase 1/2
AMD-dry, GA (RPE) – Lineage	Phase 1/2
AMD-dry, GA (RPE) – Luxa	Phase 1/2
AMD-dry, GA (RPE from iPSC) – NEI	Phase 1/2
AMD-dry, GA (RPE on scaffold) – Regen Patch	Phase 1/2
RP, Usher (retinal progenitors) – jCyte	Phase 2
RP (CD34+ stem cells) – UC Davis	Phase 1/2

Note: Some trials listed may have been paused and/or the sponsors are seeking partners to continue their trials.

PRPH2 Workshop with the Nixon Visions Foundation

On March 29–31, 2023, the Foundation Fighting Blindness hosted the inaugural PRPH2 and Associated Retinal Diseases Workshop in La Jolla, California, which brought together over 110 scientists, patients, family members, and industry professionals.

Mutations in PRPH2 lead to autosomal dominant retinitis pigmentosa, pattern dystrophies, and central areolar choroidal dystrophy. The workshop was a collaboration of the Foundation, the Nixon Visions Foundation, and the Shiley Eye Institute at the University of California, San Diego. Dr. Claire Gelfman, chief scientific officer at the Foundation, and Drs. Radha Ayyagari and Shyamanga Borooah, of Shiley Eye Institute, served as meeting co-chairs.

The goals of the workshop were to: 1) engage and build the scientific and patient PRPH2 community, 2) share knowledge, and 3) launch a PRPH2 funding initiative, which will be funded by the Nixon Visions Foundation and managed by the Foundation Fighting Blindness. The workshop featured a combination of scientific research talks, patient perspectives, and ample community-building opportunities. Various workshop materials can be found on the Foundation's website, and a peer-reviewed publication discussing the proceedings of the workshop is in preparation.

Image Description: Participants from the PRPH2 Workshop standing outside together.

In partnership with the Nixon Visions Foundation, the Foundation Fighting Blindness has launched the first call for submissions to the 'PRPH2 and Associated Retinal Degenerations Program.' Focusing on research gaps identified by a PRPH2 subcommittee, this program will fund three-year grants for each of the next three years. The Foundation anticipates making six awards of up to $500,000 each. The first awards will begin in spring 2024.

Foundation's Reach Inspires Partnership for Nixon Family

Image Description: The Nixon Family: Janine, Brandon, and their two children, Cameron and Corinne.

Janine Nixon spent more than 15 years wondering about the "disturbances" in the vision in her left eye.

"My optometrist sent me to a retinal doctor who found that I had a spot on each of my retinas," she says. "But he didn't think it was anything serious and told me not to worry about it."

Janine's vision loss was so gradual that she just lived with it. That is, until 2020, when one day, she realized she'd lost most of the central vision in her left eye. A new doctor opted to do genetic testing, which pointed to an inherited retinal disease (IRD) caused by a mutation of the PRPH2 gene.

She and her husband, Brandon, set out to learn everything they could about PRPH2-related diseases. But now, they no longer had to wonder because simple online searches provided them with plenty of information. And a lot of it was connected by a common thread.

"One of the things we noticed as we were doing our research was that the Foundation Fighting Blindness kept coming up—as a funder of research, as a sponsor of research," Janine said. "We were like, 'Wow. The Foundation seems to be the place to go.'"

But the Foundation did more than provide information. For the Nixons, who had recently formed a family foundation for various philanthropic efforts, the Foundation Fighting Blindness became an essential partner.

"Each one of the paths we would go down, we would ultimately get to where the Foundation was involved in it somewhere," Brandon said. "That's when we reached out to them to help us figure out the best path for our efforts."

With guidance from and collaboration with the Foundation, the 'PRPH2 and Associated Retinal Degenerations Program' was born. The program kicked off with the in-person 'PRPH2 and Associated Retinal Diseases Workshop,' hosted in partnership with the Foundation and Shiley Eye Institute at the University of California, San Diego, held in late March of 2023.

Among IRD patients, mutations of PRPH2 are relatively common, but they can cause more than one disease. With any disease, particularly ones that are misunderstood, it's easy to feel like you're the only one who has it. Both Brandon and Janine said it was exciting to be with people with similar conditions and to meet researchers and doctors working on treatments and cures for those conditions.

"There were breakouts where attendees got to mingle with each other and get to know each other," Janine said. "They might have been familiar with someone's work, but now they were meeting them in person. And patients were there, and they got to meet these people who are working on this gene and on these diseases. And those patients got to meet other people going through the same thing they are going through. It was really meaningful for the patients."

The workshop is just the beginning of the program the Nixons have planned with the Foundation. In partnership, the two entities have issued requests for proposals from labs around the world to fill some of the gaps in understanding PRPH2 and have received almost 30 responses.

In addition to the ongoing partnership, the Nixons have made significant gifts to the Foundation and do not hesitate to encourage other people to do the same.

"We feel that the Foundation has just got the pulse of what's going on in the world of inherited retinal diseases," Janine said. "And they're a really great partner for figuring out how to most effectively direct your funds with confidence."

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

The Foundation's sponsored Genetic Testing Program and the My Retina Tracker® Registry for people with inherited retinal diseases (IRDs) continued to expand during the fiscal year 2023. These programs and their resultant data allow the Foundation to collaborate with patients, eye care specialists, academic investigators, and life science companies to drive IRD-focused research.

Since its launch in 2017, more than 15,000 people with IRDs have received Foundation-sponsored genetic testing. The My Retina Tracker Panel screens for mutations in inherited retinal disease genes using a comprehensive 351-gene panel. Foundation-sponsored genetic counseling is available with physician referral to help patients and families understand the medical and familial implications of their results. This program benefits the patient, medical, and research communities by 1) increasing access to genetic testing for adults and children affected by IRDs; 2) allowing eye care providers to order IRD genetic testing to improve patient care; and 3) enhancing genetic data collected in the My Retina Tracker Registry which can facilitate and expedite impactful IRD research studies through de-identified data sharing and recruitment support. The Foundation is deeply committed to patient privacy and never shares personally identifying data.

More than 25,000 people are now active participants in the My Retina Tracker Registry. This program is designed to give patients and their eye care specialists the opportunity to share health data to advance IRD research. The large cohort of genetically tested individuals has become increasingly valuable as a source for de-identified research data. Registry members collectively serve as a research ready cohort that has consented to receive recruitment notifications sent by the Foundation on behalf of qualified investigators.

The Foundation's Registry team keeps Registry members up to date on the latest IRD research, including the Foundation's many research-focused activities. During fiscal year 2023, over 140,000 emails were sent to Registry members to announce research studies, clinical trials, and educational opportunities such as focus groups, gene-specific workshops, podcasts, and National Chapter sponsored webinars.

"This year, we were pleased to support the research efforts of a team at the National Eye Institute who are examining the variant data from our Registry with the goal of reclassifying variants, which will improve the diagnostic utility of gene panels in the future," said Todd Durham, PhD, senior vice president of clinical and outcomes research at the Foundation. "What this means for our community is that more IRD patients will have conclusive test results and opportunities to participate in research, including clinical trials."

The Foundation would like to thank the many adults, families, and healthcare providers who participate in the My Retina Tracker Registry and Genetic Testing Program. These programs would not be possible without their important contributions.

The Foundation also greatly appreciates the support of its partners for helping drive the growth and success of the Registry:

- The Anschutz Foundation
- The Chatlos Foundation
- The George Gund Foundation
- Hope In Focus
- Nixon Visions Foundation
- Rudolph Williams Charitable Trust
- Alnylam Pharmaceuticals
- Coave Therapeutics
- Janssen Pharmaceutical Companies of Johnson & Johnson
- Parexel
- Restore Vision
- SparingVision
- Spark Therapeutics

Genetic Testing Program

- Applied Genetic Technologies Corporation (AGTC) (now Beacon Therapeutics)
- Blueprint Genetics
- The George Gund Foundation
- Hope in Focus
- InformedDNA
- Janssen Pharmaceutical Companies of Johnson & Johnson

Image Description: Gene Testing Panel.

A Proven Global Leader

On May 24, 2023, the Foundation hosted a global webinar providing research updates from Israel. In his opening remarks, David Brint, board chair, said the Foundation's partnership with the Israeli retinal disease research community began more than 15 years ago.

In 2013, the Foundation and Israeli researchers envisioned an initiative to genetically screen all people in Israel with an inherited retinal disease (IRD). With funding from the Foundation, the Israel Inherited Retinal Disease Consortium (IIRDC) was formed in 2014 to undertake the project.

Researchers in Israel are also developing a variety of IRD therapies, many of which were reviewed in the webinar.

David emphasized the Foundation's role as a global leader in driving research for IRDs and bringing experts together from around the world to work collaboratively. The Foundation is also leading an international campaign to raise funds and engage families. Laura Fietta serves as the International Chair for the Victory for Vision campaign.

Laura Fietta is known as methodical and meticulous. When her daughter Alessia was diagnosed with a retinal disease ten years ago, that thoroughness led her straight to the Foundation Fighting Blindness.

"Not long after that, I got in touch with the Foundation when I realized that they were the world leaders in retinal disease," the London native says. "I just surely feel that the Foundation's reach and focus has been a complete game changer in terms of accelerating research."

And now, Laura is all in on helping the Foundation accelerate its ability to raise funds in the UK and around the world. She will soon be taking her place as an international trustee, a first for the Foundation.

Alessia's retinal disease is caused by the CRB1 genetic mutations, and her condition has been described as RP but is closest to retinal dystrophy, Laura says.

"That is one of the problems we face with these diseases," says Laura. "How to categorize them correctly. What I like about the Foundation is that they concentrate on each gene and take each gene seriously on its merits."

For now, Laura says Alessia recently did well on the UK standardized tests known as GCSEs. But she doesn't see well in dim light, and her peripheral vision is lacking.

"She goes to a typical school, and actually, most people don't know that she has a blinding condition," says Laura. "She just continues as if she doesn't have vision loss. And along with that comes stress. But thankfully, her condition is stable at the moment."

Meanwhile, Alessia's mom is on a mission. In June 2022, she organized a reception in London for patients and potential donors to the Foundation. Earlier this year, she helped organize a round table discussion in London for scientists, charity executives, and patients. They discuss foundations with an interest in vision loss to focus on and how best to build relationships. They've set a goal of $2.5 million dollars by December of 2025 and, so far, have commitments for $1 million.

"In terms of reaching out to other foundations, it's hard, and we're just still building relationships," she says. "Even just getting them to meet with us is difficult. But we are building relationships with other, like-minded foundations because we recognize that when we work together, we can accomplish great things."

She says it's satisfying to be involved in something where she feels like she can make a difference.

"It's a truly interesting area, and we are at a very exciting time in history," says Laura. **"You can sense that we're getting there, and we're close. But time is of the essence—because every day makes a difference to patients who are losing their sight."**

Image Description:
Laura Fietta and her daughter Alessia posing outside.

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

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

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RD FUND

The Retinal Degeneration Fund (RD Fund) was created with a dual mission: To increase the number of therapies approved and, in the process, provide the Foundation with an alternative source of revenue to further its mission.

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

A Message from the Chair and Managing Director

The Retinal Degeneration Fund (RD Fund) has played a pivotal role in advancing the Foundation's mission by innovating new therapies for retinal diseases while fostering and founding new companies to achieve this goal. Despite a challenging macroeconomic environment for the biotech sector, FY23 was another year of opportunity for the RD Fund. The RD Fund strengthened its independent Board of Directors with the appointment of four new directors and bolstered its portfolio with three new investments, including, for the first time, in the dry AMD space.

At its core, world-class science is a cornerstone of the RD Fund and its investment strategy. At the heart of the RD Fund's stakeholder base are highly successful philanthropists and business leaders who've helped fuel the construction of a retina-centric funding ecosystem. The RD Fund's unique approach to venture philanthropy leverages a collection of resources, including donor capital alongside the Foundation's deep scientific knowledge, global relationships, its Clinical Consortium, My Retina Tracker® Registry, Scientific Advisory Board, and significant outside funding from a growing group of investment partners. To strengthen the coordination of these resources, including for the scientific review of new investment opportunities, the RD Fund hired venture analyst Alicia Kemble, PhD in FY23. Dr. Kemble came to us most recently as a neuroscientist at Hoffmann La Roche, where she served on the due diligence team in Roche's division of Neuroscience & Rare Disease Therapeutics.

Central to its success is the RD Fund's governing body — an independent Board composed of eleven Directors who bring significant diverse scientific, clinical, and financial expertise to company evaluation and development. We are very fortunate to have a seasoned ophthalmology leader, Adrienne Graves, PhD, serving as our Board Chair to help lead the RD Fund and to advance companies with innovative technologies that aim to prevent, treat, or cure retinal and macular degenerations.

Many of our board members are renowned scientists and clinicians who have previously been supported by the Foundation and are anxious to give back to further the mission. We are happy to report that in this fiscal year, we added four remarkable members: Jean Bennett, MD, PhD, Cathy Bowes Rickman, PhD, Jose-Alain Sahel, MD, and Mark Blumenkranz, MD.

Dr. Bennett, F. M. Kirby, Professor of Ophthalmology at the Perelman School of Medicine at the University of Pennsylvania, is a pioneer in gene therapy for retinal diseases. Dr. Bennett's laboratory developed the first FDA-approved gene therapy for use in humans, which treats a rare form of blindness, and she was elected a member of the National Academy of Sciences in 2022. Dr. Bowes Rickman is a highly accomplished Professor at Duke University, specializing in the molecular, cell and pathobiology of AMD and the evaluation of novel therapeutic targets for its treatment. Dr. Sahel is a distinguished professor and Chairman of the Department of Ophthalmology at the University of Pittsburgh School of Medicine. Dr. Sahel, an accomplished inventor, has received numerous honors and awards and is a co-founder of multiple biotechs, including SparingVision, a clinical-stage genomic medicine company. He also founded and directed the world-class eye center in Paris, Institut de la Vision, from 2008 to 2020. Dr. Blumenkranz serves as the HJ Smead Professor Emeritus in the Department of Ophthalmology at Stanford University, and is co-director of its Ophthalmic Innovation program, and brings a wealth of R&D, venture, and governance expertise to the RD Fund's Board.

Over the past five years, the RD Fund has leveraged more than $500 million of non-Foundation funds into the field, including anchor donations from the Gordon and Llura Gund Foundation and the Manning Family Foundation. To date, the RD Fund has deployed over $78 million across fourteen investments in companies thus far in the U.S. and in Europe. These companies are working on a range of promising technologies and therapeutic targets, including gene therapy, RNA therapies, neuroprotection, and optogenetics. These companies include Amber Bio, Atsena Therapeutics, CheckedUp, Nacuity, Nayan Therapeutics, NVasc, Opus Genetics, Perceive Biotherapeutics, ProQR, SalioGen, SparingVision, Stargazer Pharmaceuticals, Vedere Bio, and Vedere Bio II.

The RD Fund continues to partner with a growing and impressive list of more than 45 top-tier venture firms and strategic partners, including large investors such as Atlas Venture, Deerfield, Abingworth, PBM Capital, Andreessen Horowitz, and Johnson & Johnson. To date, outside investors have committed over $700 million towards the RD Fund's portfolio companies. This outside capital represents well over 8-fold additional investment dollars alongside the RD Fund.

We've added three new investments in this fiscal year, which are co-funded by a group of over ten experienced venture firms.

One of the key RD Fund strategies is to diversify our portfolio to include candidate therapeutics for dry AMD and geographic atrophy, and in this fiscal year, the RD Fund made its first two investments in the area of dry AMD.

The first of these investments was in Perceive Bio, a clinically staged company developing novel treatments leading with a gene therapy candidate intended to mitigate over-regulation of the complement system for dry AMD and geographic atrophy. The company is also developing novel therapies in neuroprotection with applications in glaucoma and retinitis pigmentosa.

The second investment in FY23 in dry AMD was in NVasc, an early-stage company developing a therapeutic angiogenesis strategy for the treatment of retinal ischemia associated with the continuum of AMD. NVasc was founded by a team of highly regarded scientists led by Dr. Napoleone Ferrara. Dr. Ferrara received a Lasker award for leading the discovery of VEGF and, subsequently, the development of LUCENTIS® and AVASTIN® for the treatment of neovascular AMD, among other indications. NVasc is developing a large molecule to prevent the loss of the blood supply and expansion of atrophic regions for the treatment of dry AMD and geographic atrophy.

The RD Fund also made an investment in Amber Bio, which you can read more about in this report, an early-stage company developing a unique RNA editing platform, capable of targeting multiple mutations in one gene. This allows a single therapy to be applied to multiple mutations, rather than one therapy for each mutation which is what the previous technology would allow. Amber Bio is first applying its technologies toward inherited retinal diseases that arise from a diverse set of mutations.

Before we conclude, we must express our gratitude to former RD Fund Chair, Warren Thaler, for his dedication and leadership since the RD Fund's inception. We also thank the RD Fund's and the Foundation's Board of Directors, the Foundation's Scientific Advisory Board, and its mission-focused donor base for leading us to this place — a place that we firmly believe is one of the most exciting fields scientifically, clinically, and in biotechnology.

Thank you for your continued support.

Adrienne Graves, PhD
Chair, RD Fund

Rusty Kelley, PhD, MBA
Managing Director, RD Fund

The RD Fund has expanded its portfolio in its first five years to include the following active investments:

Amber Bio is developing a new gene editing modality that uses multi-kilobase RNA writing or splice editing technique to expand the range of treatable pathogenic variants, applicable to inherited retinal diseases.

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

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

NVasc is leading the development of an early-stage large molecule for the treatment vascular and avascular forms of age-related macular degeneration (AMD) and geographic atrophy (GA).

Opus Genetics combines unparalleled insight and commitment to patient need with wholly owned programs in numerous orphan retinal diseases.

Perceive Bio is developing novel therapeutics to prevent the largest causes of irreversible blindness with lead programs in dry AMD and neuroprotection.

SparingVision is developing a novel gene therapy approach for the treatment of inherited retinal diseases such as retinitis pigmentosa.

SalioGen Therapeutics is focused on developing therapies for more patients with inherited diseases that are beyond what is addressable with current technologies, initially focusing on inherited macular disorders and inherited lipid disorders.

Successful exits include Vedere Bio and CheckedUp, which were acquired by Novartis and Rockbridge respectively. ProQR is in active discussions to sell their Ophthalmology assets. Nayan, Stargazer, Lookout Therapeutics and Vedere Bio II underwent solvent dissolution.

The RD Fund 1 Investment Financial Summary:

Assets	June 30,2022	June 30,2023
Funds Committed to Date	$53,958,000	$61,142,363
Reserves for Future Funding for RDF Portfolio Companies	$19,011,000	$3,000,000
Funds for Future Investments	-	$8,826,775
Total Assets	**$72,969,000**	**$72,969,138**

The RD Fund 2 Investment Financial Summary:

Assets	June 30,2022	June 30,2023
Funds Committed to Date	$12,000,000	$17,550,000
Reserves for Future Funding for RDF Portfolio Companies	$8,000,000	$9,600,000
Funds for Future Investments	$24,908,000	$20,908,133
Total Assets	**$44,908,000**	**$48,058,108**

RD Fund Invests in Amber Bio to Advance New RNA-Editing Platform

The RD Fund recently invested in Amber Bio, an early-stage San Francisco-based startup developing a novel RNA editing platform to target larger sections of a gene and therefore correct more disease-causing variants at once. This approach enables the editing of multiple genetic mutations within a single disease indication, thereby expanding the addressable patient population. The latest capital raise will be used to advance this technology for treating an undisclosed retinal indication. Many previous editing platforms have addressed only single mutations, necessitating a drug for each pathogenic variant. Amber has identified novel CRISPR-based editing tools capable of making a full range of edits, potentially enabling the correction of hundreds to thousands of different variants.

Amber Bio is led by co-founders Jacob Borrajo, PhD, CEO, and Basem Al-Shayeb, PhD, CTO. Borrajo received his PhD at the Broad Institute of MIT and Harvard and is the inventor of the initial splice editor licensed to Amber Bio from MIT. He holds six patents related to gene editing, previously co-founded two startups, and is also a Y Combinator alumnus. He has worked on several projects across industry and academia, ranging from VLP-based delivery of CRISPR-Cas systems to novel RNA measurement technologies. Al-Shayeb came from the lab of Nobel Laureate Dr. Jennifer Doudna at UC Berkeley and has authored publications in top journals, including Nature, Science and Cell. He holds 19 pending and issued patents on gene editing, and his research has been highlighted in numerous articles and was recognized in Forbes' 30 under 30 for Science in 2021.

"We are thrilled to team up with RD Fund and the Foundation Fighting Blindness in building therapeutics to overcome retinal disorders," says Jacob Borrajo, CEO and co-founder at Amber Bio. "This partnership brings world-renowned experts in retinal biology and retinal therapeutic development to join in Amber Bio's mission."

The $26M series seed financing for Amber Bio was co-led by Playground Global and a16z, the health venture arm of Andreessen Horowitz. Lilly Ventures, Hummingbird VC, and Pillar VC also participated in the round alongside the RD Fund.

Image Description:
Amber Bio Co-Founders Jacob Borrajo, PhD, CEO, and Basem Al-Shayeb, PhD, CTO
(photo credit: *Business Wire*).

"We are pleased to join a strong, diverse syndicate to support Amber's skilled leadership team and their ability to design and improve a novel system for rewriting RNA," says Rusty Kelley, PhD, managing director at the RD Fund. "The retina transcriptome that includes larger genes with many disease-causing variants is ripe for accurate, efficient, and durable RNA targeting, and with reduced toxicity that may result from genomic editing."

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