# WE'RE CHANGING HOW THE WORLD TREATS BLINDING RETINAL DISEASES

Carolyn Covington

## RD FUND

Outcomes & Outlook Report
Year Ending 2021

# RAPIDLY DRIVING RESEARCH LEADING TO PREVENTIONS, TREATMENTS AND CURES FOR RETINAL DISEASES

## Purpose

The RD (Retinal Degeneration) Fund was established in 2018 to serve the mission of the Foundation Fighting Blindness to rapidly drive research toward preventions, treatments and cures for the entire spectrum of retinal degenerative diseases—including retinitis pigmentosa, macular degeneration, and Usher syndrome. Together, these blinding conditions affect more than 200 million people globally.

## Background

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

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


## The RD Fund Approach

The RD Fund is part of the Foundation's strategy for adapting to a rapidly changing environment with many more academic research projects ready for translation. The success of this model is based on leveraging our funding with other investment firms and strategic partners that allows us to accelerate more opportunities.

A bit different

# INNOVATIVE APPROACH TO INVESTING IN NOVEL OPPORTUNITIES

Venture philanthropy provides a way for donations to work like investments and donors to be more involved by funding highly visible activities to achieve the mission of a charity or nonprofit. The RD Fund aims to deliver life-changing solutions for individuals with retinal degenerative diseases today, while also creating a pipeline of next-generation and novel therapeutic opportunities for tomorrow. The RD Fund is flexible in how it structures investments leveraging equity, debt, royalty, and/or project co-funding. All proceeds of this high-impact philanthropic vehicle are returned back to the Foundation to provide resources to further its mission.



RD Fund

# FACTS OF THE FUND

**Focuses** primarily on companies with programs that are in clinical testing or can be in less than 18 to 24 months

**Uses** a variety of investment strategies including convertible debt, equity, royalties, and/or project-based co-funding

**Deploys** a geographically agnostic approach

**Targets** initial investment allocations ranging between $2 and 5 million with appropriate reserves

**Leverages** the breadth and depth of the Foundation's knowledge, global relationships and resources, including the Clinical Consortium, My Retina Tracker® Registry, Scientific Advisory Board, and outside funding from co-investors

**Provides** returns when a portfolio company exits (company is sold or becomes a publicly traded company) and funds go back to support the Foundation's mission


**Jacque Duncan, MD**

RD Fund Board Director

Foundation Fighting Blindness
Scientific Advisory Board Chair

RD Fund

# PORTFOLIO SUMMARY

The RD Fund has invested globally in multiple companies, including internally conceived startups working on a range of promising technologies and therapeutic targets, including gene therapy, RNA therapies, neuroprotection and optogenetics.

## Gene Therapy & Editing

| Atsena Therapeutics | Lookout Therapeutics | Opus Genetics | SalioGen |
|---|---|---|---|
| *AAV-based gene therapy* | *TBD* | *Ocular gene therapy technologies* | *Gene coding* |

## Readthrough & RNA Therapies

***ProQR Therapeutics***

*USH2A variant specific antisense oligonucleotide RNA therapy*

## Pharmacotherapy & Neuroprotection

| Nacuity Pharmaceuticals | Nayan Therapeutics | SparingVision SAS | Stargazer Pharmaceuticals |
|---|---|---|---|
| *Variant agnostic oral small molecule antioxidant* | *Variant agnostic antisense oligonucleotide neuroprotectant* | *Neuroprotective variant agnostic gene therapy* | *Oral visual cycle modulator* |

## Optogenetics

| Vedere Bio[1] | Vedere Bio II |
|---|---|
| *Variant agnostic optogenetic platform* | *Photoswitch optogenetic technologies for vision* |

## Digital Technology

***CheckedUp[2]***

*Specialty technology platform designed for point-of-care patient engagement*

[1] Vedere Bio was acquired by Novartis in October 2020 [2]CheckedUp was acquired by Rockbridge in December 2021



Venture Philanthropy

# THE SECRET WEAPON FOR UNLOCKING BIOMEDICAL RESEARCH'S FULL LIFE-CHANGING POTENTIAL

By Gordon Gund and Paul Manning

*The piece was first published on June 4, 2021, in Philanthropy News Digest's PhilanTopic Blog.*

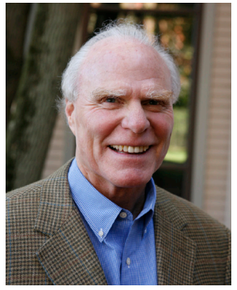
As we mark a year into the COVID-19 pandemic, much reflection is taking place around "lessons learned" across all sectors. In the biomedical research space, we've seen science answering the pandemic's urgent call with the delivery of safe and effective vaccines at miraculous speed to combat the COVID-19 virus. The mRNA technology used in some of the vaccines itself has broad implications for future treatments of a variety of diseases and is a clear indication of how far science has evolved in a short period of time. With the necessary funding, imagine what treatments and cures can be unlocked for other viruses, cancer and restoring vision.

Public funding for basic research has long come from the National Institutes of Health, but the U.S. is an outlier among other advanced economies in the small amount of funding it provides for the translational research that is necessary to convert basic science into tangible patient treatments.

While more public funding for biomedical research at the critical clinical trial stage is needed in the U.S., it is going to take public, private and philanthropic dollars to ensure biomedical research for promising treatments and cures doesn't die on the vine. Federal programs such as the Cancer Moonshot, state-level initiatives like the California Institute for Regenerative Medicine, and promising pending legislation for BioBonds, aimed at providing private sector loans to companies developing novel treatments across the spectrum of disease and disability, are helpful but leave a void. There needs to be a third leg to stabilize the public and private efforts, and we believe that third leg is philanthropy.

As successful entrepreneurs and venture investors, we see our donations as investments in the mission of a nonprofit organization. We each have a personal calling to the mission of the Foundation Fighting Blindness—as someone who has experienced loss of sight from retinitis pigmentosa as a young adult and another who raised two sons with vision impairment caused by Stargardt disease. Our collective personal experiences provide a keen understanding of what it is like to be a patient or loved one waiting on life-changing treatments. That's why we invest in the mission of the Foundation Fighting Blindness.

For 50 years, thanks to the generosity of donors, the Foundation Fighting Blindness has successfully funded the field of retinal degeneration in search of treatments and cures for the entire spectrum of inherited retinal diseases (IRDs) and dry age-related macular degeneration (AMD). Together, these blinding conditions affect more than 200 million people globally. As long-term supporters of this research, we applaud the success to date, but realize that it is not enough. The key discoveries made in the academic labs need to make it into the hands of industry-led therapy developers to get through clinical testing and over the finish line at



(From left to right) **Gordon Gund** and **Paul Manning**

*Gordon Gund is the Chairman & CEO of Gund Investment Corporation. In 1970, Gordon lost his sight from retinitis pigmentosa and in 1971, he co-founded with his wife Lulie and others the Foundation Fighting Blindness to drive the research to find treatments and cures for retinal degenerative diseases. Gordon continues to be a major donor to the Foundation Fighting Blindness and RD Fund.*

*Paul Manning is the Chairman and CEO of PBM Capital. An entrepreneur with thirty years of experience in the healthcare industry, he founded PBM Capital in 2010. Paul is a major donor to the Foundation Fighting Blindness and RD Fund – both of his sons were diagnosed with Stargardt disease.*

the FDA. But a gap in funding often prevents this progress. In fact, in the Foundation's case, the science is now outpacing the funding.

A pioneering strategy to bridge this funding gap was developed when the Foundation created the Retinal Degeneration Fund (RD Fund), a nonprofit, pure play venture philanthropy investment vehicle. This first-of-its-kind investment vehicle provides an opportunity for donations to function like investments to accelerate the technical aspects of the mission and enhance the financial goals of the Foundation. The RD Fund allowed us to be more intimately involved by funding highly visible activities in biotech startups and spin outs.  We have taken concepts and techniques from our venture capital finance and business management experience and applied them to our philanthropic goals of accelerating the progress on treatments and cures, while positioning the Foundation for long-term sustainability. The resulting RD Fund was launched in late 2018 with $72M under management, and our family foundations together contributed significant capital to the Foundation to launch the RD Fund.

In just three years, the first Fund is now 90 percent committed with nine investments plus reserves. To date, this invested capital has attracted an additional $400 million in capital from institutional co-investors. Fund 1 has even produced its first exit with the sale of Vedere Bio to Novartis for $280 million ($150 million upfront) after just 14 months of operations. This transaction allowed the Foundation to plug a financial gap in its long-range science spending plan AND roll over significant funds to seed Fund 2. This is the magic of venture philanthropy—the Foundation not only gets to "play it again," but it has a multiple on top of that to "pay it forward."

Based on the initial returns of the RD Fund, both in therapeutic development as well as financially, the Foundation Fighting Blindness has launched fundraising efforts for RD Fund 2, targeting at least $75M in new capital via philanthropic donations. Together, we have committed an additional $30M from our respective family foundations, because we believe in this organization and the venture philanthropy model.

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

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

# BY THE NUMBERS

as of December 2021

**RD Fund 1** (all funds committed)

**$72,969,138**

Total Investment Assets

**$54,708,310**

Funds Committed to Date

**$18,258,709**

Reserves for Future Funding of Portfolio Companies

**RD Fund 2**

**$41,562,993**

Total Investment Assets

**$12,000,000**

Funds Committed to Date

**$23,000,000**

Reserves for Future Funding of Portfolio Companies

**$6,562,993**

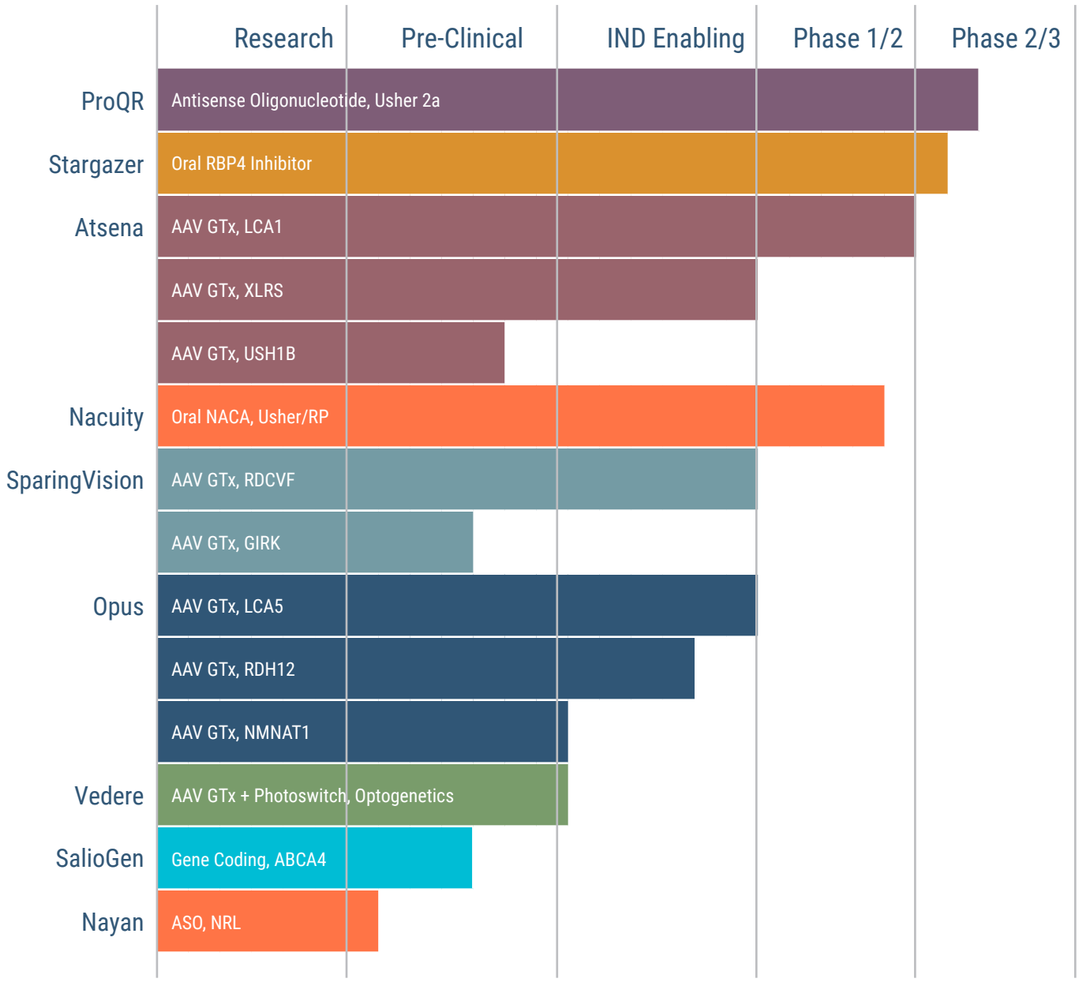
Funds Available for Future Investments

RD Fund Portfolio Companies

# THERAPEUTIC PIPELINE

as of December 2021

- ProQR
- Stargazer
- Atsena
- Nacuity
- SparingVision
- Opus Genetics
- Vedere Bio II
- SalioGen
- Nayan






ATSENA THERAPEUTICS

Atsena Therapeuticsis a clinical-stage gene therapy company focused on bringing the life-changing power of genetic medicine to reverse or prevent blindness. Atsena is developing novel gene therapies, including a Phase 1/2 clinical program for Leber congenital amaurosis (LCA1), XLRS and Ush1b based on research from Dr. Shannon Boye's lab at the University of Florida.

## Highlights

- Founded Atsena to develop novel gene therapies for IRDs.
- LCA1 Phase 1/2 trial expected to complete enrollment in 2022.
- Preclinical programs advancing.
  - IND-enabling studies for XLRS program.
  - Dose finding study in NHP for USH1B program.
- New facility construction nearing completion with move-in in 2022.

## Key Milestone/Inflection Points

- Data from cohort 3 of LCA1.
- IND filing for XLRS.
- USH1B pre-GLP non-human primate dose-ranging studies.

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|---|---|
| **Year Founded** | 2020 |
| **Location** | Durham, NC |
| **Stage at Investment** | Preclinical |
| **CEO** | Patrick Ritschel, MBA |
| **No. of Employees** | 25 FTE |
| **Website** | atsenatx.com |
| **Approach/Delivery** | AAV-based, subretinal delivery |
| **Disease Focus** | LCA1; USH1B; XLRS |
| **Clinical Stage/Status** | Phase 1/2 for LCA1 |
| **Year of Initial Investment** | 2020 |
| **Investment** | $8.5 million |
| **Governance** | Board observer |
| **Significant Co-Investors** | Sofinnova Partners<br>Abingworth<br>Lightstone Ventures<br>Hatteras Venture Partners<br>Osage University Partners<br>The Manning Family Foundation<br>University of Florida<br>Wilson Sonsini Goodrich & Rosati |
| **Key Terms** | Series A preferred |

CheckedUp®

CheckedUp is a healthcare tech company that deploys a state-of-the-art platform into specialty healthcare facilities, including eyecare practices, across the U.S.  to actively engage patients, caregivers, and physicians in the waiting room, exam room, and at home. The Company recently launched a new telemedicine platform during the COVID-19 pandemic and is the only 100% digital, push technology platform designed for specialty point of care.

## Pre-Exit Highlights

- Point-of-care video and interactive technology drives exposure of the Foundation to patient audiences in eyecare offices by providing up-to-date information on events and key initiatives initially focused on Eyecare. Continued growth of the flagship eyecare network, with new digital offerings for providers & patients.
- Launched specialty verticals in Rheumatology, Dermatology, and in Oncology with flagship partner, Merck in 2020.  Clients include 17 of the top global pharma companies.

## Key Milestone/Inflection Points

- Company acquired by Rockbridge Growth Equity.
  - Allows existing management to grow the company.
  - $1,234,179.53 upfront to RDF; RDF 2 carries equity in the new company.

| | |
|---|---|
| **Year Founded** | 2012 |
| **Location** | New York, NY |
| **Stage at Investment** | N/A |
| **CEO** | Richard Awdeh, MD |
| **No. of Employees** | 35 FTE |
| **Website** | checkedup.com |
| **Approach/Delivery** | Healthcare technology |
| **Sector Focus** | Digital health |
| **Year of Initial Investment** | 2018 |
| **Investment** | $1.0 million |
| **Governance** | Board observer |
| **Key Terms** | Convertible note |



LOOKOUT THERAPEUTICS

Lookout Therapeuticsis a RD Fund spin-out company founded with a leading venture capital group with significant expertise in gene therapy and rare diseases.

## Highlights

- Formed company to develop novel genetic therapies for rare inherited retinal diseases.

## Key Milestone/Inflection Points

- Pending licensing activity.

| | |
|---|---|
| **Year Founded** | 2020 |
| **Location** | Charlottesville, VA |
| **Stage at Investment** | TBD |
| **CEO** | Paul B. Manning |
| **No. of Employees** | N/A |
| **Website** | N/A |
| **Approach/Delivery** | Ocular gene therapy |
| **Disease Focus** | Inherited retinal diseases |
| **Clinical Stage/Status** | Licensing discussions |
| **Year of Initial Investment** | 2020 |
| **Investment** | $5 million |
| **Governance** | Two board director seats |
| **Key Terms** | Series Seed preferred |

NACUITY PHARMACEUTICALS, INC

Nacuity is a clinical stage pharmaceutical company working on a breakthrough treatment for RP, addressing oxidative stress in the retina, which causes cell degeneration and vision loss in virtually all forms of RP. Nacuity's approach using N-acetylcysteine-amide (NACA), with its anti-oxidative properties, may benefit most people with RP, regardless of the gene mutation causing their disease. This approach is based on studies from the laboratory of Dr. Peter Campochiaro at the Wilmer Eye Institute involving oxidative stress in the retina. Dr. Campochiaro's research has been partially funded by the Foundation Fighting Blindness.

## Highlights

- Phase 1/2 trial for RP in USH presently has 36 enrolled (many on target dose of 500mg/day for almost one year well tolerated), recruiting 2–3 per week thus full enrollment (48) expected before end of 1Q 2022.
- Toxicology study and clinical protocol complete, Ethics Approval received for Australian anti cataract trial in post vitrectomy patients using NPI-002 implant.
- Completed a $16.5M Series B raise.

## Key Milestone/Inflection Points

- Australian Phase 1/2 proof of concept readouts late 2022; 6-month safety data expected March 2022, possibly enabling U.S. based RP Phase 2/3 in 2022.
- Cataract trial initiation Feb. 2022 with POC readout 3Q 2022.

| | |
|---|---|
| **Year Founded** | 2016 |
| **Location** | Fort Worth, TX |
| **Stage at Investment** | Preclinical |
| **CEO** | Halden Conner |
| **No. of Employees** | 5 FTE |
| **Website** | nacuity.com |
| **Approach/Delivery** | Oral/implanted small molecule antioxidant |
| **Disease Focus** | Retinitis pigmentosa (RP) and Cataracts |
| **Clinical Stage/Status** | Phase 2 RP/USH 80% recruited; Phase 1/2 Cataract Feb |
| **Year of Initial Investment** | 2017 |
| **Investment** | $5.5 million non-dilutive co-funding; $4 million Equity |
| **Governance** | Board seat, observer seat |
| **Significant Co-Investors** | Tamagulia Ventures |
| **Key Terms** | Series B + warrants |



NAYAN

Nayan Therapeutics is a preclinical stage company developing mutation-agnostic therapies to treat inherited retinal diseases. Nayan is developing novel small molecules that preserve cone function by down regulation of rod-specific genes, thereby potentially preserving color and central vision in patients with inherited retinal diseases. The company was founded based on research from Dr. Tom Reh's lab at the University of Washington. Dr. Reh's research has been partially funded by the Foundation Fighting Blindness.

## Highlights

- Completed a $25 million Series A funding led by RA Capital.
- Identification of antisense oligonucleotides (ASOs) that downregulate NRL transcription factor and selectively downregulate rod genes.

## Key Milestone/Inflection Points

- Identification of NRL ASOs that are homologous to both humans and non-human primates (NHPs) – necessary for extending safety and toxicity in NHPs before going into first-in-human trials.
- Expanded NHP study dose response to explore therapeutic index for rod selective knockdown and retinal stability.
- Completed 60-day dose-escalation study in NHPs with two lead ASO candidates. Pending assessment of safety & efficacy, a development candidate will be nominated 1Q 2022.

| | |
|---|---|
| **Year Founded** | 2019 |
| **Location** | Medfield, MA |
| **Stage at Investment** | Preclinical |
| **CEO** | Milind Deshpande, PhD |
| **No. of Employees** | 2 FTEs |
| **Website** | N/A |
| **Approach/Delivery** | Mutation-agnostic therapies |
| **Disease Focus** | Retinitis pigmentosa (RP) |
| **Clinical Stage** | Preclinical |
| **Year of Initial Investment** | 2019 |
| **Investment** | $1.0 million |
| **Governance** | Board observer |
| **Significant Co-Investors** | RA Capital |
| **Key Terms** | Series A preferred |

Opus Genetics combines unparalleled insight and commitment to patient need with wholly owned programs in numerous orphan retinal diseases. Its AAV-based gene therapy portfolio tackles some of the most neglected forms of inherited blindness while creating novel orphan manufacturing scale and efficiencies. Based in Raleigh, N.C., the company leverages knowledge of the best science and the expertise of pioneers in ocular gene therapy to transparently drive transformative treatments to patients.

## Highlights

- Closed $19M Series Seed on August 5, 2021, with founding investors (Manning Family Foundation and Bios Partners),
- Executed U Penn license for lead programs LCA5 & RDH12, and MEEI/Harvard license for NMNAT1.
- Key hires include Dr. Ash Jayagopal as CSO, Joe Schachle as COO, and Brian Leising as VP of CMC.
- Selection of a contract manufacturer for first three products and selected clinical CRO for LCA5 study.

## Key Milestone/Inflection Points

- Submit the LCA5 IND expected 2Q 2022.
- Submit the RDH12 pre-IND meeting package and hold pre-IND meeting expected 2Q 2022.
- Evaluation and in-license of 4th and 5th pipeline programs.

| | |
|---|---|
| **Year Founded** | 2021 |
| **Location** | Raleigh, NC |
| **Stage at Investment** | Preclinical |
| **President, acting CEO** | Ben Yerxa, PhD |
| **No. of Employees** | 3 FTEs |
| **Website** | opusgenetics.com |
| **Approach/Delivery** | Ocular gene therapy technologies |
| **Disease Focus** | LCA5, LCA13 (RDH12), LCA9 (NMNAT1) |
| **Clinical Stage/Status** | Preclinical |
| **Year of Initial Investment** | 2021 |
| **Investment** | $10 million |
| **Governance** | Two board director seats |
| **Significant Co-Investors** | Bios Partners<br>Manning Family Foundation |
| **Key Terms** | RDF led Series Seed preferred |



ProQR is a clinical stage company developing transformative RNA therapies for the treatment of severe genetic rare diseases such as Leber congenital amaurosis 10 (LCA10), Usher syndrome and other forms of retinitis pigmentosa, based on proprietary RNA repair platform technologies.

## Highlights

- In March 2021, ProQR reported positive results from a planned analysis of the Phase 1/2 Stellar trial demonstrating concordant benefit on multiple measures of vision, and ultevursen (QR-421a) was observed to be well tolerated with no serious adverse events reported.
- Following on the positive results, ProQR has initiated two pivotal Phase 2/3 trials; “Sirius” and “Celeste”. Sirius focuses on advanced patients with a primary endpoint of BCVA at month 18; and Celeste focuses on early-moderate patients with a primary endpoint of static perimetry at month 12. In both trials the first patients were dosed in December 2021.
- ProQR has started enrolling eligible patients from the Stellar study into the Helia open-label extension study, which includes multiple dose treatment for both eyes.

## Key Milestone/Inflection Points

- ProQR expects to share an update from the Helia open-label extension study of ultevursen by year end 2022.

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|---|---|
| **Year Founded** | 2012 |
| **Location** | Leiden, Netherlands & Cambridge, MA |
| **Stage at Investment** | Preclinical |
| **CEO** | Daniel A. de Boer |
| **No. of Employees** | 188 FTEs |
| **Website** | proqr.com |
| **Approach/Delivery** | Antisense oligonucleotide / intravitreal injection |
| **Disease Focus** | Genetic forms of blindness |
| **Clinical Stage** | Phase 1–Phase 3 |
| **Year of Initial Investment** | 2018 |
| **Investment** | $7.5 million |
| **Governance** | Joint development committee |
| **Significant Co-Investors** | Puiblicly traded on NASDAQ under PRQR |
| **Key Terms** | Fixed multiple ROI |

SaligoGen Therapeutics is advancing a new category of medicine termed Gene Coding, for adding nucleic acids into a patient's genome to turn on, off, or modify function of a new or existing gene(s). SalioGen's gene coding platform is based on a proprietary, first-and-only mammalian-derived enzymes in development, known as Saliogase, that uses Exact DNA Integration by Transposition (EDIT) technology. The company, initially pursuing non-viral delivery of large genes including in ophthalmology, was co-founded by serial entrepreneur, Ray Tabibiazar and former Human Genome Project researcher, Joseph Higgins, and based on the work of Howard Hughes investigator Nancy Craig.

## Highlights

- The RD Fund Board approved a $2M investment as a participant in an upsized $115M+ Series B financing.
- RD Fund and the company formed a Joint Development Committee that gives Foundation Fighting Blindness/RD Fund visibility and input into the preclinical development of the ocular programs.

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|---|---|
| **Year Founded** | 2020 |
| **Location** | Cambridge, MA |
| **Stage at Investment** | Preclinical |
| **CEO** | Ray Tabibiazar, MD |
| **No. of Employees** | 30 FTE |
| **Website** | saliogen.com |
| **Approach/Delivery** | LNP, subretinal delivery |
| **Disease Focus** | Ocular and other inherited disorders |
| **Clinical Stage** | Preclinical |
| **Year of Initial Investment** | 2021 |
| **Investment** | $2 million |
| **Governance** | Joint development committee |
| **Significant Co-Investors** | PBM Capital<br>Gordon MD<br>EPIQ Capital Group<br>Fidelity<br>T.Rowe Price<br>D1 Capital Partners<br>SymBiosis<br>The Cystic Fibrosis Foundation |
| **Key Terms** | Series B preferred |



SparingVision is a genomic medicines company, translating pioneering science into vision-saving treatments. Founded to advance over 20 years of world-leading ophthalmic research from its scientific founders at the Paris Vision Institute, SparingVision is leading a step shift in how ocular diseases are treated, moving beyond single gene correction therapies. At the heart of this is a pipeline of gene independent treatments for rod-cone dystrophies. Lead products, SPVN06 and SPVN20, address mid and late stages of retinitis pigmentosa (RP) respectively, regardless of genetic cause. RP is the most common inherited retinal disease affecting two million people worldwide.

## Highlights

- Hired Dr. Daniel Chung as Chief Medical Officer (ex Spark Tx), Dr. Deniz Dalkara as Chief Scientific Officer (part time) and Dr. Mehdi Gasmi as Chief Operations Officer.
- Acquisition and incorporation of Gamut gene & cell therapy company and new gene-therapy product SPVN20.
- Strategic collaboration with Intellia to develop genome-editing based products on up to 3 ocular targets (targeting IRDs).
- Initiation of Phenorod U.S. prospective natural history study Q4 2021 (90% completion of EU patient recruitment).

## Key Milestone/Inflection Points

- Completion of SPVN06 preclinical studies and filing of an IND with the FDA in 2022; Initiation of FIH study in 4Q 2022.
- Lead candidate selection for SPVN20.
- Intellia collaboration: selection of 2/3 targets.

| | |
|---|---|
| **Year Founded** | 2016 |
| **ocation** | Paris, France |
| **Stage at Investment** | Preclinical |
| **CEO** | Stéphane Boissel, MBA |
| **No. of Employees** | 19 FTEs |
| **Website** | sparingvision.com |
| **Approach/Delivery** | Neuroprotective gene therapy, subretinal |
| **Disease Focus** | Retinitis pigmentosa (RP) |
| **Clinical Stage** | Preclinical |
| **Year of Initial Investment** | 2016 |
| **Investment** | $14 million |
| **Governance** | Board seat |
| **Significant Co-Investors** | Bpifrance<br>La Fondation Voir & Entendre<br>UPMC Enterprises<br>4Bio Capital<br>Jeito Capital<br>Ysios Captial<br>AdBio<br>Intellia |
| **Key Terms** | Series A preferred |

STARGAZER PHARMACEUTICALS

Stargazer Pharmaceuticals is a biopharmaceutical company developing treatments for rare eye diseases, including STG-001, an oral, nonretinoid visual cycle modulator to treat Stargardt disease. STG-001 is designed to reduce blood concentrations of RBP4, a protein that delivers vitamin A to the retina. By reducing the uptake of vitamin A in the retina, researchers believe that STG-001 can potentially reduce the accumulation of retinal toxins for people with Stargardt disease and prevent retinal degeneration and subsequent vision loss.

## Highlights

- Completed a $57 million Series A financing with lead investor Novo Ventures, venBio Partners, Canaan Partners, and Pontifax Venture Capital.
- Completion of the Phase 2a study 2Q 2021.
- Phase 3 study design accepted by CHMP and FDA.

## Key Milestone/Inflection Points

- Modeling of the Phase 2a PK/PD data (and Phase 1 data in healthy volunteers) to optimize and support Phase 3 dosing selection.
- BD, partnering, and M&A discussions are ongoing.

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|---|---|
| **Year Founded** | 2018 |
| **Location** | Boston, MA |
| **Stage at Investment** | Clinical |
| **CEO** | David Meek, (ad-interim) |
| **No. of Employees** | 3 FTEs |
| **Website** | stargazerpharmaceuticals.com |
| **Approach/Delivery** | Oral small molecule<br>Visual Cycle Modulator |
| **Disease Focus** | Stargardt disease |
| **Clinical Stage** | Phase 3 ready |
| **Year of Initial Investment** | 2020 |
| **Investment** | $0.250 million with $0.5 million warrant option |
| **Governance** | Board observer |
| **Significant Co-Investors** | Novo Ventures<br>venBio Partners<br>Canaan Partners<br>Pontifax Venture Capital |
| **Key Terms** | Series A preferred |



vedere

Vedere Bio II is an emerging biopharmaceutical company utilizing novel ocular gene therapy technologies to develop a pipeline of vision restoration and vision preservation therapies for underserved indications. In October 2020, Novartis acquired Vedere Bio, an RD Fund portfolio company, leading to the formation of Vedere Bio II to continue pursuing next generation ocular gene therapies for vision restoration.

## Highlights

- Closed $77M Series A with founding investors (Atlas Venture, Mission Bio and RD Fund) and new investors (Octagon Capital, Samsara Biocapital, and Casdin Capital).
- Identified through multi-year directed evolution screens, proprietary, intravitreally-delivered AAV capsids that transduce the retinal ganglion cells, bipolar cells and photoreceptors; validated in African Green and/or Cynomolgus (1Q 2022).
- Advancement of proprietary CMC platform for potential commercialization of lead program.

## Key Milestone/Inflection Points

- Development candidate nomination for lead optogenetics program expected in 1Q 2022; IND submission for lead expected mid-2023.

| | |
|---|---|
| **Year Founded** | 2020 |
| **Location** | Cambridge, MA |
| **Stage at Investment** | Preclinical |
| **CEO** | Cyrus Mozayeni, MD, MBA |
| **No. of Employees** | 21 FTEs |
| **Website** | vederebio.com |
| **Approach/Delivery** | Ocular gene therapy technologies |
| **Disease Focus** | Retinitis pigmentosa (RP) and dAMD |
| **Clinical Stage** | Preclinical |
| **Year of Initial Investment** | 2020 |
| **Investment** | $5 million |
| **Governance** | Board observer |
| **Significant Co-Investors** | Octagon Capital<br>Samsara BioCapital<br>Casdin Capital<br>Atlas Venture<br>Mission BioCapital |
| **Key Terms** | Series A preferred |

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

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

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

With gratitude,

Warren Thaler
RD Fund, Chair



# BOARD OF DIRECTORS & MANAGEMENT

## Board of Directors

The RD Fund has an independent Board of Directors comprised of prominent investors, business executives, and clinicians to oversee the portfolio of investments, and to ensure alignment with the Foundation's mission. They are also charged with balancing risk versus return, and to ensure diversification of indications and therapies within the category.

*Warren Thaler, MBA*
Chairman
RD Fund

Retired President
Gund Investment Corporation

*David Brint*
Chairman
Foundation Fighting Blindness

Chief Executive Officer
Brinshore Development Company

*Anthony P. Adamis, MD*
Former Global Head of Ophthalmology, Immunology and Infectious Diseases, Genentech/Roche

*Eugene de Juan, MD*
Vice-Chairman
ForSight Labs

Jean Kelly Stock Distinguished Chair in Ophthalmology
University of California San Francisco

*Jacque Duncan, MD*
Professor of Clinical Ophthalmology
Beckman Vision Center, University of California San Francisco

*Adrienne Graves, PhD*
Former Chief Executive Officer
Santen Inc.

*Kelly Lisbakken*
Managing Director & Head of Biopharma Investment Banking
Wedbush PacGrow

*Jonathan Steinberg, MD*
Director
SMG Arrhythmia Center

Director
Cardiac Clinical Trials and Education
Summit Medical Group

## Management

The RD Fund's seasoned management team is deeply aligned with the Foundation's mission and actively seeks and evaluates viable and beneficial partnerships.

*Ben Yerxa, PhD*
Chief Executive Officer
Foundation Fighting Blindness and RD Fund

*Rusty Kelley, PhD, MBA*
Senior Vice President, Investments & Alliances
Foundation Fighting Blindness

*Jason Menzo*
Chief Operating Officer
Foundation Fighting Blindness

*Peter Ginsberg*
Executive Vice President Corporate Development, Chief Business Officer
Foundation Fighting Blindness

*Claire M. Gelfman, PhD*
Chief Scientific Officer
Foundation Fighting Blindness

RD FUND

**For More Information:**
RDFund.org

**Rusty Kelley, PhD, MBA**
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