WE'RE CHANGING HOW THE WORLD TREATS BLINDING RETINAL DISEASES

RD FUND

January 2021
Outcomes & Outlook Report

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

Purpose

The Retinal Degeneration Fund (RD 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. The RD Fund focuses on mission-related investments in companies with projects nearing clinical testing. The RD Fund provides equity and/or non-dilutive financing strategies for start-up and early-stage companies.

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 FUNDING LATE-STAGE RESEARCH

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

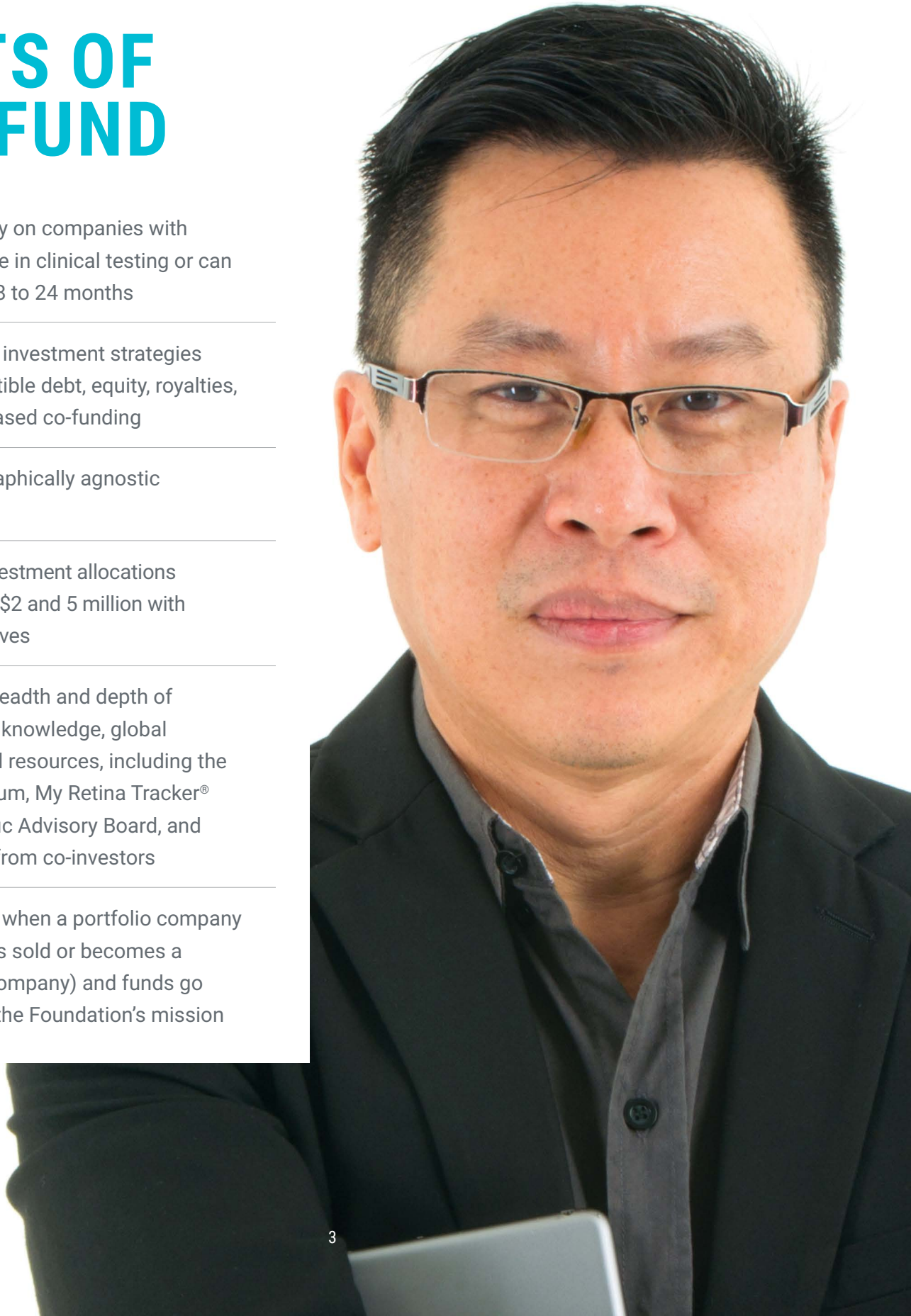
Uses a variety of investment strategies including convertible debt, equity, royalties, and/or project-based co-funding

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



RD Fund

PORTFOLIO COMPANY SUMMARY

The RD Fund focuses on companies with late preclinical to clinical stage programs. Preclinical projects are generally within 18 to 24 months of clinical testing. 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
AAV-based gene therapy (LCA1[1], USH1B)

Lookout Therapeutics
Variant specific gene therapy

Readthrough & RNA Therapies

ProQR Therapeutics
USH2A variant specific antisense oligonucleotide RNA therapy

Pharmacotherapy & Neuroprotection

Nacuity Pharmaceuticals
Variant agnostic oral small molecule antioxidant for RP[2]

Nayan Therapeutics
Variant agnostic antisense oligonucleotide neuroprotectant for RP

SparingVision SAS
Neuroprotective variant agnostic gene therapy for RP

Stargazer Pharmaceuticals
Oral visual cycle modulator for Stargardt disease

Optogenetics

Vedere Bio[3]
Variant agnostic optogenetic platform for RP and AMD

Vedere Bio II
Technologies for vision restoration

Digital Technology

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

[1] Leber Congenital Amaurosis [2] Retinitis Pigmentosa [3] Vedere Bio was acquired by Novartis in October 2020

A Success Story

RD FUND REACHES A MILESTONE

In October 2020, an exciting milestone was reached when Vedere Bio became the first RD Fund portfolio company to achieve an exit. Vedere Bio, a Cambridge, Massachusetts based biotech company, is focused on next generation optogenetic gene therapy as an approach to restore vision in patients that have lost most vision due to degeneration of photoreceptors. This technology has the potential to work regardless of the genetic cause of the disease and works by introducing a light-responsive gene into cells that do not normally respond to light, making them light-sensitive.

Vedere Bio was founded based on work primarily from the labs of Drs. John Flannery and Ehud Isacoff of the University of California, Berkeley, and technology from the University of Pennsylvania. Vedere Bio was launched in June 2019 with a $21 million equity financing led by Atlas Venture of which the RD Fund contributed $3 million.

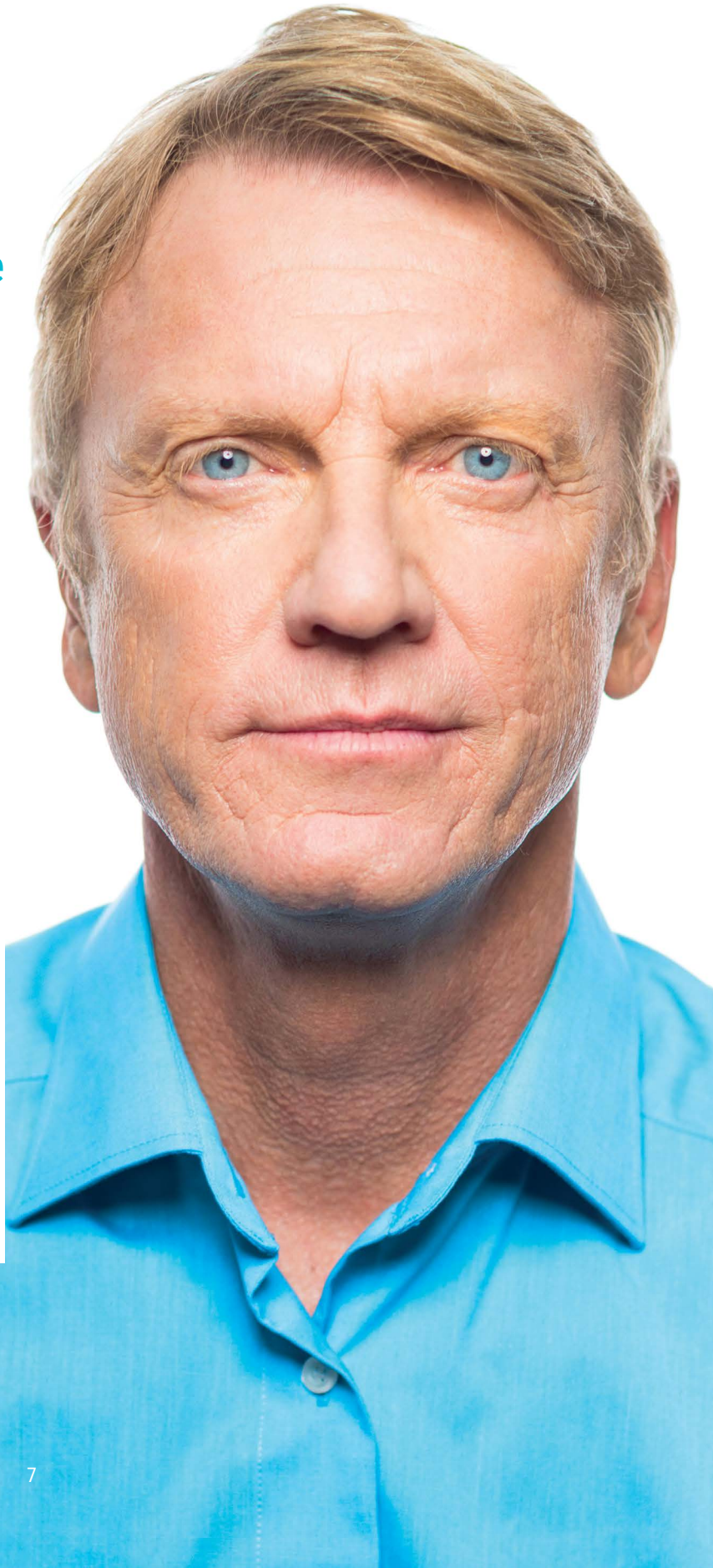
Vedere Bio's advanced technology caught the attention of industry leaders, and in October 2020, the company announced its acquisition by Novartis for $150 million in upfront payments. Including future potential milestone payments, the total deal was valued at approximately $280 million, effectively achieving a return of investment of four-fold with the upfront return and up to seven-fold pending near-term earnouts from milestone achievements.

Vedere Bio's acquisition by Novartis validates the power of the venture philanthropy model for accelerating our mission while providing for meaningful returns to support the Foundation's mission. Importantly, Novartis plans to invest significant resources to bring this technology into the clinic, and if successful, ultimately to patients in need.

An additional benefit of this transaction is that the RD Fund has also invested in the same team to form Vedere Bio II, a spinout of Vedere Bio, which is working on next generation gene therapies for retinal degenerative diseases.

(From left to right) **John Flannery,** PhD, **Ehud Isacoff**, PhD and **Cyrus Mozayeni**, MD, MBA

This successful progress by Vedere Bio is a strong proof point of the impact of venture philanthropy in achieving real and accelerated progress in the development of new therapies for patients with vision loss.



#1

Largest preclinical ophthalmic gene therapy M&A deal to date.

Top 5

One of the five largest ophthalmic gene therapy M&A deals to date.

16 Months

Fastest ophthalmic gene therapy M&A deal from company inception to acquisition.

RD Fund 1

BY THE NUMBERS

as of December 2020

$71,969,137

Total RD Fund Investment Assets

$48,283,510

Funds Committed to Date

$17,685,628

Reserves for Investments Made to Date

$65,969,138

Committed + Reserves to Date

$36,885,474

Funds Called to Date

$5,999,999

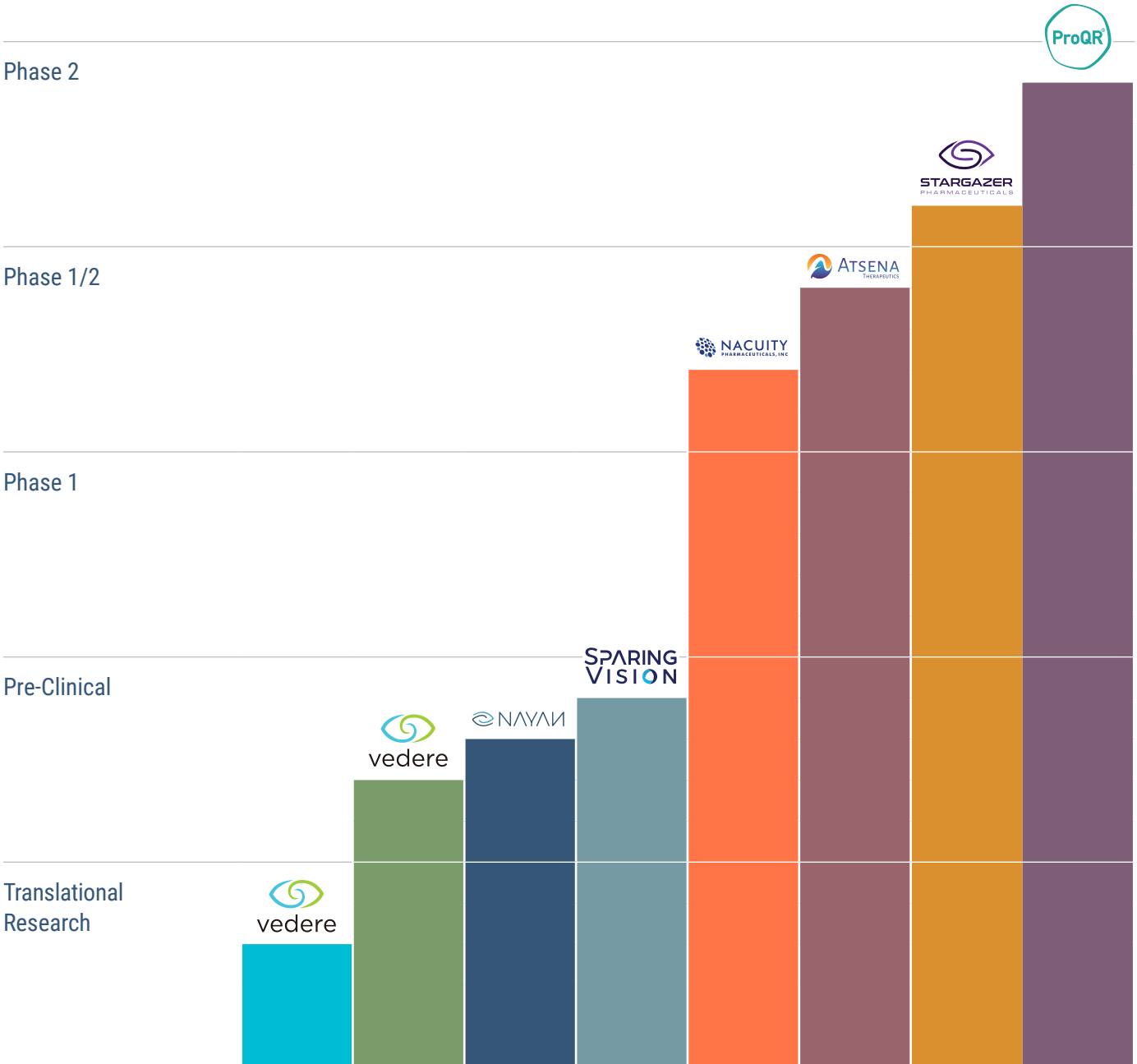
Remaining Funds for Future Investments

RD Fund 1 Portfolio Companies

THERAPEUTIC PIPELINE

as of December 2020

● ProQR (Usher) ● Stargazer (Stargardt) ● Atsena (LCA) ● Nacuity (RP)
● SparingVision (RP) ● Nayan (mutation-agnostic) ● Vedere Bio (optogenetic gene therapy)
● Vedere Bio II (gene therapy) ● Lookout (ocular gene therapy)



ATSENA THERAPEUTICS

Atsena Therapeutics is 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), based on research from Dr. Shannon Boye's lab at the University of Florida.

Year Founded	2020
Corporate Headquarters	Durham, NC
CEO	Patrick Ritschel, MBA
Website	atsenatx.com
RD Fund Vintage Year	2020
RD Fund 1 Investment	$8.5 million committed
RD Fund Oversight	Board member
Significant Co-Investors	Sofinnova Partners Abingworth Lightstone Ventures Hatteras Venture Partners Osage University Partners The Manning Family Foundation University of Florida Wilson Sonsini Goodrich & Rosati
Deal Type	Equity
Approach/Delivery	AAV-based, subretinal delivery
Disease Focus	LCA1; USH1B
Clinical Stage/Status	Phase 1/2 for LCA1

Highlights

- Founded Atsena to develop novel gene therapies for IRDs
- Acquired exclusive rights from Sanofi to a clinically staged gene therapy program targeting GUCY2D-associated LCA1, a genetic eye disease that affects the retina and is a leading cause of blindness in children
- Completed an $8.15 million Series Seed funding co-led by the RD Fund and Hatteras Venture Partners
- Completed a $55 million Series A financing led by Sofinnova Partners, with participation from new investors Abingworth and Lightstone Ventures, and alongside existing investors including the RD Fund, Hatteras Venture Partners, and Osage University Partners

Upcoming Catalysts

- Complete enrollment in the second cohort of its LCA1 Phase 1/2 trial

CheckedUp® is a healthcare technology 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. CheckedUp was ranked #279 on the 2019 Inc. 5,000 list of the Fastest-Growing Private Companies in the U.S.

Year Founded	2012
Corporate Headquarters	New York, NY
CEO	Richard Awdeh, MD
Website	checkedup.com
RD Fund Vintage Year	2018
RD Fund 1 Investment	$1.0 million committed
RD Fund Oversight	Board observer
Deal Type	Convertible note
Approach/Delivery	Healthcare technology
Sector Focus	Digital health

Highlights

- CheckedUp's point-of-care video and interactive technology drives exposure of the Foundation Fighting Blindness and its mission to patient audiences in eyecare offices by providing relevant content and up-to-date information on events and key initiatives.
- Initially focused on four specialty verticals: Eyecare (including Retina), Rheumatology, Dermatology, and Oncology (launched 1st half 2020)
- Clients include 11 of the top 15 global pharma companies
- Recently secured flagship oncology partner, Merck
- Launched new telemedicine platform called Virtual Visits for specialist healthcare providers

Upcoming Catalysts

- Launch fifth specialty vertical by end 2021
- Continue financial growth including positive EBITDA[1] and cash flow

[1] Earnings before interest, taxes, depreciation, and amortization

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

Year Founded	2020
Corporate Headquarters	Charlottesville, VA
RD Fund Vintage Year	2020
RD Fund 1 Investment	$5.0 million committed
RD Fund Oversight	Two board members
Deal Type	Equity
Approach/Delivery	Ocular gene therapy
Disease Focus	Inherited retinal diseases
Clinical Stage/Status	Licensing discussions

Highlights

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

Upcoming Catalysts

- Actively exploring licensing opportunities

NACUITY PHARMACEUTICALS, INC

Nacuity is a clinical stage pharmaceutical company working on a breakthrough treatment for RP related to Usher syndrome, 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.

Year Founded	2016
Corporate Headquarters	Fort Worth, TX
CEO	Halden Connor
Website	nacuity.com
RD Fund Vintage Year	2017
RD Fund 1 Investment	$7.5 million committed
RD Fund Oversight	Board member & Board observer
Significant Co-Investors	Tarnagulla Ventures
Deal Type	Equity; project co-funding
Approach/Delivery	Small molecule, orally administered
Disease Focus	Retinitis pigmentosa (RP)
Clinical Stage/Status	Phase 2; currently enrolling patients

Highlights

- Completed preclinical toxicology programs and filed an Investigative New Drug application with the U.S. FDA
- Completed a Phase 1 clinical trial in healthy volunteers in Australia in 2019
- Initiated a Phase 1/2 trial in Australia in 2020 focused on the treatment of RP in patients with Usher syndrome

Upcoming Catalysts

- Reporting on the first round of safety data from the ongoing Phase 2 trial by Q4 2021 and reporting efficacy data by mid to late 2022
- Targeting Series B financing in 2021

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.

Year Founded	2019
Corporate Headquarters	Medfield, MA
CEO	Milind Deshpande, Ph.D.
RD Fund Vintage Year	2019
RD Fund 1 Investment	$1.0 million committed
RD Fund Oversight	Board observer
Significant Co-Investors	RA Capital
Deal Type	Equity
Approach/Delivery	Mutation-agnostic therapies
Disease Focus	Retinitis pigmentosa (RP)
Clinical Stage/Status	Preclinical

Highlights

- Completed a $25 million Series A funding led by RA Capital

Upcoming Catalysts

- Validation of biology with a lead candidate by mid-2021

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.

Year Founded	2016
Corporate Headquarters	Leiden, Netherlands & Cambridge, MA
CEO & Founder	Daniel de Boer
Website	proqr.com
RD Fund Vintage Year	2018
RD Fund 1 Investment	$7.5 million committed
RD Fund Oversight	Two joint development committee members
Significant Co-Investors	Publicly traded on NASDAQ under PRQR
Deal Type	Project co-funding, fixed multiple ROI
Approach/Delivery	Antisense oligonucleotide / intravitreal injection
Disease Focus	Usher syndrome and other forms of non-syndromic RP
Clinical Stage/Status	Phase 2/3; currently enrolling patients

Highlights

- Announced positive findings from a planned three-month interim analysis of its Phase 1/2 Stellar trial of QR-421a in adults with Usher syndrome and non-syndromic retinitis pigmentosa (nsRP) due to USH2A exon 13 mutations
- Presented positive data from the ongoing InSight extension trial of sepofarsen for LCA10, in which patients from the completed Phase 1/2 trial were offered treatment in their second eye

Upcoming Catalysts

- Completion of enrollment in Illuminate Phase 2/3 trial of sepofarsen in Q1 2021; additional data from Phase 1/2 InSight extension study to be reported in H2 2021
- Completion of enrollment in the Stellar Phase 1/2 clinical trial of QR-421a in Q4 2021 and reporting of Phase 1/2 interim analysis expected in H1 2021
- Completion of Phase 1/2 Aurora trial of QR-1123 for autosomal dominant retinitis pigmentosa (adRP) and reporting of initial single-dose cohorts of this program in 2021

SparingVision is a biotechnology company focused on the discovery and development of innovative therapies for the treatment of blinding inherited retinal diseases. SparingVision is developing a gene-independent treatment for retinitis pigmentosa, the most common inherited retinal disease. SPVN06 is designed to prevent the degeneration of cone photoreceptors leading to blindness. There is currently no therapy to treat all genetic forms of this rare retinal disease that leads to blindness and affects nearly 2 million people worldwide.

Year Founded	2016
Corporate Headquarters	Paris, France
President & CEO	Stephane Boissel, MBA
Website	sparingvision.com
RD Fund Vintage Year	2016
RD Fund 1 Investment	$14.7 million committed
RD Fund Oversight	Board member
Significant Co-Investors	Bpifrance La Fondation Voir & Entendre UPMC Enterprises 4Bio Capital Jeito Capital Ysios Capital
Deal Type	Equity
Approach/Delivery	Neuroprotective gene therapy, subretinal
Disease Focus	Retinitis pigmentosa (RP)
Clinical Stage/Status	Preclinical

Highlights

- Completed 44.5€ million financing round, securing several new leading global investors, including 4BIO Capital, UPMC Enterprises, Jeito Capital and Ysios Capital; in addition, current investors, the RD Fund and Bpifrance, participated in the financing
- Appointed the SparingVision Chairman of the Board, Stéphane Boissel, as Chief Executive Officer
- Received European Orphan Designation for its drug candidate, SPVN06, dedicated to inherited retinal dystrophies

Upcoming Catalysts

- Completion of preclinical studies and filing of an Investigational New Drug application with U.S. FDA in late 2021

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.

Year Founded	2018
Corporate Headquarters	Boston, MA
CEO	Gary Sternberg, MD, MBA
Website	stargazerpharmaceuticals.com
RD Fund Vintage Year	2020
RD Fund 1 Investment	$250,000 committed, with $500,000 warrant option
RD Fund Oversight	Board observer
Significant Co-Investors	Novo Ventures venBio Partners Canaan Partners Pontifax Venture Capital
Deal Type	Equity/Warrants
Approach/Delivery	Small molecule, orally administered
Disease Focus	Stargardt disease
Clinical Stage/Status	Phase 2; currently enrolling patients

Highlights

- Completed a Phase 1 safety trial of STG-001 in healthy volunteers
- Initiated a Phase 2a clinical trial of STG-001, an indirect visual cycle modulator, in Stargardt disease patients
- Completed a $57 million Series A financing with lead investor Novo Ventures, venBio Partners, Canaan Partners, and Pontifax Venture Capital

Upcoming Catalysts

- Completion of Phase 2a trial in early 2021
- Initiation of Phase 2b/3 trial in mid-2021

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. The newly formed Vedere Bio II, Inc., will be referred to as Vedere going forward.

Year Founded	2020
Corporate Headquarters	Cambridge, MA
CEO & Founder	Cyrus Mozayeni, MD, MBA
Website	vederebio.com
RD Fund Vintage Year	2020
RD Fund 1 Investment	$500,000 committed
RD Fund Oversight	Board observer
Significant Co-Investors	Atlas Venture Mission BioCapital
Deal Type	Equity
Approach/Delivery	Ocular gene therapy technologies
Disease Focus	Retinitis pigmentosa (RP) and age-related macular degeneration (AMD)
Clinical Stage/Status	Preclinical

Highlights

- Launched Vedere Bio II with Vedere Bio's founders, team and facilities to advance its pipeline
- Secured funding from investor syndicate of Atlas Venture, Mission BioCapital and the RD Fund

Upcoming Catalysts

- Completion of IND readiness studies in late 2022

Introducing

RD FUND 2

Based on the success and impact of RD Fund 1, the Foundation Fighting Blindness has launched fundraising efforts for RD Fund 2. In addition to making new investments, RD Fund 2 has the flexibility to make follow-on investments in RD Fund 1 companies. RD Fund 2 will build on the diversity of the overall portfolio, including novel strategies based on modality (genetic approaches, neuroprotection, optogenetics, and cell replacement), time of intervention, gene-specific and gene-agnostic approaches to help address over 300 identified inherited retinal diseases. RD Fund 2 is also able to support technologies such as devices, large and small molecule delivery, diagnostics, telemedicine, and healthcare IT.

Active fundraising is underway for major gift donations for RD Fund 2, which already includes a $15 million anchor investment by The Manning Family Foundation.

"We are able to see the impact of venture philanthropy in achieving real progress in the development of new therapies for patients with vision loss. For the millions of patients globally living with retinal degenerations, the advancements funded by the RD Fund provide concrete solutions for the future."

Warren Thaler, MBA
Chairman
RD Fund

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

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

Jason Menzo
Chief Operating Officer
Foundation Fighting Blindness

Peter Ginsberg
Executive Vice President
Corporate Development

Chief Business Officer
Foundation Fighting Blindness

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

RD FUND

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