

**Foundation Fighting Blindness**  
***Insights Forum* Transcript**  
**January 29, 2021**

**Chris Adams, Vice President, Marketing & Communications:**

Good afternoon and welcome to the Foundation Fighting Blindness quarterly Insights Forum. I am Chris Adams, the Vice President of Marketing & Communications at the Foundation, and we appreciate you joining us for today's call.

Before we get started, I would like to briefly review a few logistical details for the call. Currently, all participant lines are in listen-only mode with no video. Today's conference is being recorded and is available in closed captioning. To activate the closed captioning, please select the closed captioning option located at the bottom of the Zoom interface. Please note that on today's call, our speakers do have their video live, however, all of their comments will be provided verbally and there are no slides.

If you are using a screen reader, please be aware that the controls are at the bottom of the Zoom interface. This control bar may collapse when it is not in use. If you prefer to prevent the controls from auto-hiding, go to settings within the Zoom platform, select accessibility and then select "always show meeting controls". It might be helpful to maximize your window and navigate by using the tab key. Additional buttons and settings are available by pressing the ALT key.

During the call, you may ask questions through the Q&A and Chat features, or by sending an email to [info@fightingblindness.org](mailto:info@fightingblindness.org). We will address questions toward the end of the call during the Q&A session, at which time, additional instructions for asking questions will be provided.

I would now like to turn the call over to Jason Menzo.

**Jason Menzo, Chief Operating Officer:**

Thank you, Chris, and good afternoon everyone. Thank you for joining us today! My name is Jason Menzo, and I am the Chief Operating Officer here at the Foundation Fighting Blindness.

I would like to welcome you to our quarterly *Insights Forum*, which highlights the latest developments here at the Foundation Fighting Blindness and within the broader retinal disease community.

We have a great line up for you today.

First, I will highlight some of our recent activities and events focused on increasing community engagement, fundraising and public awareness of our mission;

I will then hand it over to Peter Ginsberg, who recently joined us as Executive Vice President of Corporate Development and Chief Business Officer. Peter will provide a brief snapshot of his newly created role, followed by a summary of our financial performance through December 31, 2020;

Then our CEO, Dr. Ben Yerxa, will share some reflections on the importance of our strategic partnerships as we move forward in 2021; and

We will then conclude with an update on our venture philanthropy initiative, the Retinal Degeneration Fund, or the RD Fund, led by Senior Vice President of Investments & Alliances, Dr. Rusty Kelley.

Following our formal remarks, we will have a question-and-answer period, and at that time, Chris will repeat the instructions on how to ask questions. As Chris mentioned, this call is being closed captioned, and a replay and fully accessible transcript will be available on our website in the weeks ahead.

If you have any feedback related to our accessibility standards or other suggestions for this call, or the Foundation in general, please reach out to us at the email address: [info@FightingBlindness.org](mailto:info@FightingBlindness.org).

This year the Foundation is celebrating our 50<sup>th</sup> anniversary, and to recognize this important milestone, we are launching many new initiatives and highlighting the fact that we truly are winning the fight, together as one team. You may have noticed that we've been using the tagline "Together, We're Winning" in our communications, and today, I'd like to share some of the additional ways we are honoring and celebrating this legacy of winning over the last 50 years.

One of the Foundation's most important roles is supporting and educating eye care professionals who are out in the community diagnosing and managing the care for patients with IRDs. Last fall, our Professional Outreach team hosted several educational webinars. The first was entitled "Practical Management of Inherited Retinal Diseases", featuring keynote speaker, Dr. Rachel Huckfeldt from Mass Eye and Ear. That was followed by a panel session on "Low Vision Resources and Rehabilitation." These courses were very well received, with nearly 200 eye care professionals participating live, and many more who have participated online in the recorded sessions. Though the material is targeted for eye care professionals, the replays are available on the Foundation's website for anyone to review.

In late October, we hosted our fall Virtual VisionWalk. As you would imagine, we needed to pivot due to the ongoing pandemic, so we merged 15 walks that were scheduled to take place all over the country in person into one National Virtual VisionWalk. We also had 58 teams register and fundraise in areas where we don't typically host walks which is fantastic! In total, we surpassed our fundraising goal and raised more than \$1 million towards our mission! A tremendous accomplishment for a virtual event.

In December, we hosted our second "Music to our Eyes" livestream, which featured a concert and conversation with a special guest who has a connection to our mission. The purpose of this series is to raise awareness, especially among new audiences who may not be familiar with the Foundation.

This most recent episode featured Kodi Lee, a singer-songwriter and pianist, who rose to fame after winning the 14th season of the reality tv show *America's Got Talent*, back in 2019. Kodi was born with optic nerve hypoplasia, causing him to become legally blind, and was also diagnosed with autism at an early age.

A replay of this special event can be found on our YouTube and Facebook pages. Stay tuned for details on our next episode which is planned for later this spring.

Another series of popular events here at the Foundation are the National Chapter Vision Webinars. These webinars are presented free of charge through the support of The Chatlos Foundation and other partners.

This past Saturday, we hosted a Gene Therapy webinar, that attracted more than 1,600 registrants. This webinar featured a panel discussion of the many ways gene therapies can be used to address retinal degenerative diseases, as well as the latest updates on gene therapy research and clinical trials.

This webinar, which is available on our website for replay, was moderated by our Senior Director of Scientific Outreach, Ben Shaberman, with well-respected panelists, including Dr. John Flannery of UC Berkeley and scientific co-founder of Vedere Bio; Dr. Shannon Boye of the University of Florida and scientific co-founder of Atsena Therapeutics; and Dr. Pete Adamson, Vice President of Ophthalmology for Janssen Pharmaceuticals.

These webinars are hosted by various local Foundation Fighting Blindness chapters. We are very fortunate to have a strong network of more than 40 volunteer-led chapters across the country that host a variety of events critical to our success.

This year we plan to build on the success of our existing chapters and grow – both in terms of the number of active chapters, as well as the number of engaged members in each chapter. We are proactively investing to make this happen.

In this current world of virtual events, the Foundation continues to search for innovative & creative ways for our community to connect. As we look towards the spring and summer of 2021, the pandemic continues to impact our plans. Our number one priority is keeping the Fighting Blindness community healthy and safe. And so, we have decided to host our signature spring events virtually, including our first ever virtual gala.

Please join us on Rare Disease Day, Sunday, February 28th, for our virtual gala, which is called Hope from Home: A United Night to Save Sight, featuring celebrity emcee and Saturday Night Live alum, Kevin Nealon.

From the comfort of your own home, experience a virtual party packed with comedy, inspiration, and special musical performances, all supporting the Foundation Fighting Blindness and our mission.

This event will feature musical entertainment from award-nominated recording artist, Lachi, singer-song writer Charlie Kramer, a silent and live auction, and the opportunity to interact and move between different party rooms with activities including tastings, music and games, health and wellness, sports, and science.

This night is going to be even more special, because we will also announce and present our highest research honor, the Llura Liggett Gund Award.

We want to sincerely thank our Chairs, Dr. Alice Cohen and Dr. Jonathan Steinberg, and the amazing event committee for putting together an evening that you won't want to miss! All the details are on our website, [FightingBlindness.org](http://FightingBlindness.org).

As I wrap up today, I'm pleased to have the opportunity to introduce one of our newest team members, Peter Ginsberg, who joined us last month in the new role of Executive Vice President of Corporate Development and Chief Business Officer.

Peter is leading our financial plans and strategy, along with driving business transactions, strategic planning, and new activities aimed at creating novel revenue streams across the Foundation and RD Fund. Peter has worked extensively within ophthalmology, inspired by his long-time mentor, who has retinitis pigmentosa.

I'm pleased to turn the call over to Peter.

**Peter Ginsberg, EVP, Corporate Development and Chief Business Officer:**

Thank you, Jason. I am excited to be part of the Foundation Fighting Blindness, having spent 25 years in the rare disease field, first on the financial side, where I led investments in promising young companies, and more recently as head of corporate development and strategy for two publicly traded rare disease companies ... all along with a keen interest in ophthalmic diseases, as Jason noted.

Throughout that time, I experienced how important foundations can be in the development of new treatments and in bringing together key stakeholders such as patients, clinicians and companies. The Foundation is doing all of these things now, and I look forward to helping accelerate the achievement of our mission.

Now, I'd like to provide a brief summary of our financial position. As a reminder, the Foundation operates on a July-to-June fiscal year, so our 2021 fiscal year will end on June 30, 2021. Our audited financial statements for fiscal 2020 are available on the Foundation's website in the About Us section under Financial Reporting.

For the first six months of fiscal 2021, unrestricted revenue was approximately \$9.8 million against expenses of \$6.2 million.

We are tracking to our overall budget plan for fiscal year 2021, which includes targeted revenue of \$21.2 million against operating expenses of \$14.5 million, with greater than 70% of those expenses going directly to mission related efforts. Importantly, the Foundation expects to spend roughly \$20 million this fiscal year on research projects that we hope and believe will lead to preventions, treatments and cures for people affected by retinal degenerative diseases.

In addition to the key fundraising events that Jason mentioned, we work closely with leading and emerging companies in our field that provide financial support for Foundation initiatives. These collaborations provide companies with an opportunity to engage with patients, clinicians, other companies and also experts in our field, so we can accelerate retinal disease research.

Our corporate partners for 2021 currently include: AGTC, the Allergan Foundation, Apellis, Astellas, Biogen, Genentech, Janssen, MeiraGTx, Spark and Two Blind Brothers.

I'd like to highlight one of our important sponsorship renewals. We are encouraged by the progress made by MeiraGTx, which is developing gene therapies for X-linked retinitis pigmentosa, RPE65-deficiency and achromatopsia in clinical trials...much of the work in partnership with Johnson & Johnson. At the American Academy of Ophthalmology annual meeting last November, MeiraGTx presented Phase 1/2a clinical data in a small number of XLRP patients indicating that statistically significant vision improvement was seen and that improvement was sustained for one year after treatment. These early data are promising and we look forward to this gene therapy's further development.

These corporate sponsorships are critical, however, they are a small part of our overall annual fundraising efforts. Individual donations and fundraising events provide the bulk of the funds we raise each year. We are grateful for the strong and broad support of our community that you represent. I'd now like to turn the call over to our CEO, Dr. Ben Yerxa.

**Dr. Ben Yerxa, Chief Executive Officer:**

Thank you, Peter. It's great to have you on the team! Good afternoon everyone and thank you for joining us on our quarterly update call. Since we are at the beginning of the new calendar year, it's a good time to step back and highlight a theme central to accomplishing our mission. And that's "teamwork".

One of the best ways we can leverage and multiply the impact of the Foundation in our community is by forging partnerships. We can accelerate our results by collaborating with peer organizations, community leaders and other visionaries in the field.

As Peter highlighted, corporate partnerships have enabled the Foundation to invest in cutting-edge science directed towards a variety of promising research opportunities. We are grateful for this support.

We also have many other types of collaborations. Let me share a few examples.

We work with other organizations and non-profits to secure research funding for specific conditions and the development of novel treatments.

For example, we are partnering with Dr. James Free and his wife, Carole, along with other restricted funds, in the Free Family AMD Research Program, which is providing \$3 million in funding for new research projects related to the development of therapies for age-related macular degeneration.

Sofia Sees Hope, has partnered with the Foundation to support therapy development and genetic testing. This nonprofit was founded by Laura Manfre and Charles Priebe to generate awareness, raise funds for research, and provide outreach, support and education to those affected by Leber congenital amaurosis, or LCA, and other inherited retinal diseases.

Two Blind Brothers, a mission-driven clothing company, founded by Bradford and Bryan Manning, donates 100 percent of profits to fund research through a partnership with the Foundation. We have also collaborated with Two Blind Brothers in creating and hosting our Music to Our Eyes series that Jason mentioned.

We partner with medical experts and industry players to generate information relevant to the broad community, such as natural history studies and genetic testing.

For example, we are currently conducting the Pro-EYS study for people with retinitis pigmentosa, or RP, caused by mutations in the gene EYS. These multi-year studies are critical for understanding the impact any therapy may have on the natural progression of disease. These efforts help accelerate the development of treatments. The findings from the studies are published and widely disseminated so that we can share what we learn with therapy developers from around the world. Our combined goal is to boost and accelerate development for all commercial and academic researchers.

These studies require significant investment, with more than \$5 million required to run just one study. We want to do more of these studies over time - so this is an area in which partners can help accelerate our progress.

We have multiple collaborators supporting the multi-year Pro-EYS study, including the Jaeb Center for Health Research, a nonprofit clinical research organization, Duke Reading Center, Casey Reading Center, Blueprint Genetics, InformedDNA, Emmes, Kari Branham, University of Michigan, Stephen Daiger, University of Texas and Robert Hufnagel, at the NIH and National Eye Institute.

We are also partnered with Blueprint Genetics and InformedDNA to offer My Retina Tracker Program, an open access, no-cost genetic testing program. The genetic testing is provided through Blueprint Genetics, a leader in the field of clinical genetic testing of rare inherited diseases. Genetic counseling services are provided by InformedDNA, leveraging their full-time staff of lab-independent, board-certified genetics specialists. We are also grateful to receive very generous financial support from the George Gund Foundation to help run these important programs.

We look for opportunities to partner with organizations that enable us to leverage expertise and education to broaden our scope in an efficient manner.

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

We also kicked off a new partnership late last year with Odylia Therapeutics, an independent nonprofit organization whose mission is to move new drug therapies for rare disease from research into the clinic. We are teaming up with their experts to hold a four-part webinar series on preclinical and translational research. This series focuses on genetic technologies for blinding diseases and provides actionable preclinical development information to researchers looking to advance their ideas into the clinic to help patients.

In addition to the partners I've just mentioned, we have many other teammates working towards our mission, including our Board of Directors, Scientific Advisory Board, Clinical Consortium, RD Fund Board and Foundation staff. We are honored to work with so many amazing individuals and organizations.



As we celebrate our 50<sup>th</sup> anniversary year, the message “Together, We’re Winning” captures the essence of our many constituents, including our national organization, the robust scientific community, our chapters, volunteers and donors all coming together to fight the diseases that cause blindness.

When it comes to leveraging the power of partnerships, one notable and important example is our RD Fund.

The 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.

The initial creation of the RD Fund was made possible by another essential partnership with the donors who contributed to the Gordon and Llura Gund Family Challenge.

It’s part of our strategy for adapting to a rapidly changing environment with many more academic research projects ready for translation into clinical testing and commercialization. Running clinical trials is expensive and complicated. In order to spread the investment and risk, the RD Fund approach is based on leveraging our funding with other investment firms and strategic partners that allows us to accelerate more opportunities than we could fund on our own – a “hyper-accelerating” effect.

I am pleased to have on the call today my colleague, Dr. Rusty Kelley, who is our Senior Vice President of Investments & Alliances. With a strong scientific and financial background and expertise in clinical development and venture funding, Rusty joined our team three years ago to help coordinate our efforts around the RD Fund.

Rusty has been instrumental in driving the success of the RD Fund and the critical role we are playing in helping get more potential treatments and cures to our community. Today, he is going to provide a snapshot of the Fund’s portfolio companies and our next steps to leverage the momentum we have created. Rusty, please go ahead.

**Dr. Rusty Kelley, Senior Vice President, Investments & Alliances:**

Thank you, Ben, and good afternoon everyone. I appreciate the opportunity to provide you with an update on the activities of the RD Fund and our portfolio companies.

As Ben described, the RD Fund serves a key role in bringing donors and innovators together. In simple terms, the RD Fund uses a venture philanthropy model that brings to bear financial resources, the resources of the Foundation, and surrounds itself with like-minded investment firms to select and support promising companies.

Venture Philanthropy is a type of impact investment that takes concepts and techniques from venture capital finance and business management and applies them to achieving philanthropic goals, in our case via mission related investments that aim to provide both clinical and financial returns to the Foundation.

This approach leverages the breadth and depth of the Foundation's knowledge, global relationships and resources including the Clinical Consortium, My Retina Tracker® Registry, our Scientific Advisory Board, and outside funding from co-investor partners.

Let me share with you a few points on how the Fund works.

We focus primarily on companies with programs that are in clinical testing or can be in the clinic in less than 18 to 24 months.

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

Our initial investment allocation ranges between \$2 and 5 million, with appropriate reserves to provide each company additional funding as needed.

All proceeds that the RD Fund receives back over time from these investments are returned to the Foundation to provide resources to further our mission. For example, the Fund would receive funds back when a portfolio company is sold or becomes a publicly traded company.

The RD Fund has an independent Board of Directors with representatives from the Foundation Fighting Blindness and other experienced community members, who bring significant scientific, clinical and financial expertise to the team. We are very fortunate to have an experienced investment executive, Warren Thaler, as our RD Fund Board chair. Warren is a long-time key supporter of the Foundation and has served as a Foundation board member for many years.

Warren is joined on the RD Fund Board by:

David Brint, Chairman of the Foundation;

Dr. Jacque Duncan - UCSF Ophthalmologist and Chair of the Foundation's Scientific Advisory Board;

Dr. Jonathan Steinberg - Chair of the Foundation's Research Oversight Committee,

Dr. Adrienne Graves – former CEO of Santen;

Kelly Lisbakken - Managing Director and the Head of Biopharma investment banking at Wedbush PacGrow;

Dr. Gene de Juan - Vice Chair of Foresight Labs, serial inventor, entrepreneur and ophthalmologist; and

Importantly, one of the Foundation's founders and the RD Fund's anchor donor, Gordon Gund, is an honorary and active Board member.

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, pharmacotherapy or neuroprotection, optogenetics, and digital technologies.

We have made ten investments to date in the following companies: Atsena Therapeutics, CheckedUp, Lookout Therapeutics, Nacuity, Nayan Therapeutics, ProQR, SparingVision, Stargazer Pharmaceuticals, Vedere, and Vedere Bio II.

The ten investments total nearly \$43 million in currently committed capital, and with approximately \$23 million in capital reserves for the existing portfolio, we have committed 90% of our \$72 million under management for the Fund.

The RD Fund continues to partner with a growing and impressive list of top tier venture firms and strategic partners, such as Atlas Venture, RA Capital, Abingworth, Hatteras Venture Partners, Paul B. Manning Capital and Novartis.

To date, outside investors have committed over \$235 million towards the RD Fund portfolio companies. This outside capital represents well over 5-fold additional investment dollars alongside the RD Fund.

There are four companies in our portfolio conducting clinical trials that are currently enrolling patients – Atsena Therapeutics, Nacuity, ProQR and Stargazer. I'll provide a brief overview of each of these companies.

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.

Atsena has raised \$8.15 million Series Seed funding led by the RD Fund, and Hatteras Venture Partners. And they just completed a \$55 million Series A financing led by Sofinnova Partners, with participation from Abingworth and Lightstone Ventures, alongside existing investors.

Atsena is currently enrolling the second cohort of its LCA1 Phase 1/2 trial.

Nacuity is a clinical stage pharmaceutical company working on a breakthrough treatment for RP by 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 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 - work that was partially funded by the Foundation.

Nacuity has completed preclinical toxicology programs and filed an IND, (Investigative New Drug application) with the U.S. FDA.

They completed a Phase 1 clinical trial in healthy volunteers in Australia in 2019 and initiated a Phase 1/2 trial in Australia in 2020 focused on the treatment of RP in patients with Usher syndrome.

Nacuity expects to report on the first round of safety data from the ongoing Phase 2 trial by Q4 2021 and report efficacy data by mid to late 2022.

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

ProQR has 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 due to USH2A exon 13 mutations – a program co-funded by the RD Fund.

They plan to complete enrollment in the Stellar Phase 1/2 clinical trial of QR-421a at the end of 2021 and report Phase 1/2 interim analysis in the first half of 2022.

Stargazer Pharmaceuticals is a biopharmaceutical company developing treatments for rare eye diseases. Their lead candidate STG-001, an oral nonretinoid visual cycle modulator to treat Stargardt disease, 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.

Stargazer has completed a Phase 1 safety trial in Australia of STG-001 in healthy volunteers and they are currently enrolling a Phase 2a clinical in Stargardt disease patients.

They completed a \$57 million Series A financing in the spring of 2020 with lead investor Novo Ventures, venBio Partners, Canaan Partners, and Pontifax Venture Capital.

They are targeting completion of the Phase 2a trial in early 2021 and expect to initiate a Phase 2b/3 trial in the middle of 2021.

This level of clinical activity is truly exciting and indicates the near-term potential of research that the Foundation has been funding.

Now moving on to the other portfolio companies, I'll provide a brief summary of each company's focus.

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.

Lookout Therapeutics is a new RD Fund portfolio company founded with a leading venture capital group with significant expertise in gene therapy and rare diseases. Lookout is working to bring in promising technologies in the IRD and/or dry AMD space.

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, partially funded by the Foundation.

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.

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 the 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 upfront. Including future potential milestone payments, the total deal was valued at approximately \$280 million, effectively achieving a return of investment of over four-fold with the upfront return and potentially greater than seven-fold pending near-term payments 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 Vedere team and alongside the same investors to form Vedere Bio II, a spinout of Vedere Bio, which is working on next generation gene therapies for retinal degenerative diseases.

Based on the success and impact of RD Fund 1, we have launched fundraising efforts for RD Fund 2.

Our aim is to build on the diversity of the overall portfolio, including novel strategies based on modality, time of intervention, gene-specific and gene-agnostic approaches to potentially help address as many of the over 300 identified inherited retinal diseases as possible.

Although the RD Fund 2 will primarily focus on Therapeutics, it will also consider supporting technologies such as devices, large and small molecule delivery, diagnostics, telemedicine, and healthcare IT that advance the Foundation's mission.

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 look forward to updating you on these efforts. We have just published our first RD Fund Outlooks and Outcomes Report, which provides detailed non-confidential information about the Fund and our portfolio companies. You can find this report and additional information about the RD Fund at our website, [RDFund.org](http://RDFund.org).

To summarize, the RD Fund is a critical part of the Foundation's efforts 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.

We are grateful to our donors, investment partners and innovative portfolio companies for creating opportunities that can make a difference for our community. Thank you for your attention, and I'll now turn the call back over to Jason.

**Jason Menzo, Chief Operating Officer**

Thank you, Rusty, for sharing that update. It is truly amazing to see the progress being made by these companies and we look forward to great things to come.

We will now open the call to take your questions and comments. Chris, please provide the instructions for asking questions.

**Chris Adams, Vice President, Marketing & Communications:**

Thanks, Jason. There are several methods for asking questions.

First, you may access the Q and A and Chat features located at the bottom of the Zoom control bar and type in your questions.

Second, you can ask questions verbally. To do so, please select the hand raising function on the menu bar at the bottom of the Zoom interface and we will provide you with instructions to unmute yourself.



And third, if you joined by phone for today's call, you can press Star 9, to raise your hand. Pressing Star 6 will mute and unmute your line. You may also submit your questions via email at [info@fightingblindness.org](mailto:info@fightingblindness.org).

Please note that if there are questions that we aren't able to answer on today's call due to time constraints, we will follow up with you directly via email over the next week or two.

**Jason Menzo, Chief Operating Officer**

Excellent, and we do have a few questions that have already been chatted in and the first one I am going to direct to Amy. We have two questions regarding particular areas of the IRD landscape that folks are curious about whether there are any research updates we can share. The first is Bardet-Biedl syndrome and the second is a type of LCA NMNAT1. Please address any updates that you are familiar with on those two areas of the RD landscape.

**Dr. Amy Laster, Vice President, Science and Awards Programs**

Thank you. Good afternoon everyone. To address the Bardet-Biedl syndrome, it is a complex disorder that effects many parts of the body, including the retina. Typically, individuals with this syndrome will have a retinal degeneration that's similar to retinitis pigmentosa. There are researchers at the University of Iowa that have been establishing a pre-clinical gene therapy program for two forms of Bardet-Biedl syndrome. These studies are still in animals but they are advancing towards translating their studies into human clinical trial.

With regards to LCA that's due to mutations in the NMNAT1 gene, this is a common cause of LCA that accounts for about 5% of the cases. It's an early onset recessive disease. The gene is relatively small. This makes it an attractive candidate for gene augmentation therapy. There are researchers currently at the Mass Eye and Ear who are developing an AAV mediated gene augmentation therapy as a potential treatment. These studies are also being tested in animals and the recent data from a subretinal injection using AAV that carries the normal copy of the NMNAT1 showed it rescued retinal structure and function. This is data that's very important as a first step to demonstrate proof of concept for a therapy to treat patients with this form of LCA.

There's also some other preclinical research that's going on that focuses on how the protein that's encoded by this gene works and this is to allow researchers to

identify other targets as potential treatment strategies in order to treat this form of disease.

Towards the question of the gene therapy treatment for LCA that's out now, LUXTURNA, that's for specific mutations due to RPE 65, so it would only be effective for individuals with mutations for RPE 65 and not for patients that have NMNAT1 mutations.

**Jason Menzo, Chief Operating Officer**

Thank you so much, Amy. I will take the opportunity to introduce you. Dr. Amy Laster is our Vice President of Science and Awards program. She oversees all of the many award programs and funding of the researchers across the globe that we are so proud to work with. We are very happy to have her join us on this Insights Forum. Thank you very much for that. We will possibly have more questions for you as the Q and A session continues.

The next question was about the pronunciation of Vedere Bio, so I wanted to make sure that I answer that question.

A great sort of aspirational question is one I am going to direct to you Ben. Which projects are you most excited about in 2021? There's obviously a lot that we do, between the RD fund, My Retina Tracker, our grants and awards, and all the companies spinning out. There's a lot of different things to be excited about. As the CEO, what is the one project or initiative you are most excited about?

**Dr. Ben Yerxa, Chief Executive Officer:**

That's a good question and a tough one because I think there is so much to be excited about. I think high on my list is optogenetics because when you look at the project developed by Vedere Bio and acquired by Novartis, it has tremendous potential to help a wide variety of people suffering from late stage disease. The data looks really really good. When you see data like that and it doesn't just look good to people in the field, but people who are looking for breakthrough therapeutics and are willing to put that kind of money down on something, it's really validating to the power of this kind of therapy. So I think that's certainly high on my list and then after that, there are just so many exciting programs where clinical data is starting to be generated. I think any program where there's really good animal data for gene augmentation, and a solid therapy in the clinic - stay tuned for these results as they are going to come in rapidly in the next one to two years.

**Jason Menzo, Chief Operating Officer**

Let me follow up on another question about optogenetics. I recognize at this point the work is preclinical. The question is, do we have any sense what type of vision the patient might expect from optogenetics when it makes it into the clinic and to people?

**Dr. Ben Yerxa, Chief Executive Officer:**

It's hard to make these predictions, but we do know at the level of the animal models where we are going into a blind mouse, a mouse with no photo receptors, they have been able to restore visual acuity to the level of a fraction of the centimeter. So it's not perfect and it's unknown about the speed of the light detection, there maybe a blurring effect, and it may require some optimization, but these animals have been able to see moving lines that are quite narrow with good contrast sensitivity and recognize objects in a dim room. Again, these are animal studies - these are mice so they can't tell us how they are seeing. It's some of the strongest data we have ever seen in the field, so we are encouraged.

**Jason Menzo, Chief Operating Officer**

A reminder - you can ask questions through a variety of different ways. We have a couple of questions that have come in about My Retina Tracker. It may be a good time for Todd to give a brief overview of My Retina tracker. Are there any updates or feedback that you can provide when genetic tests come back with an inconclusive finding?

**Dr. Todd Durham, Vice President, Clinical & Outcomes Research**

We are up to over 16, 000 members in our registry right now and over half of these have genetic testing results in their profile. We are pleased with the growth of this program. Just as a reminder - the purpose of this program is to enable researchers to contact relevant individuals for research studies, clinical trials, natural history or patient journey studies. We are satisfying requests quite a bit for researchers in the community. I encourage all of our members to keep their profiles up to date. We do use your profiles to match you with research opportunities. I also want to say that we have a number of opportunities in which we approach all of our membership to contribute information, such as the study we did last year with Retina International on the cost of illness. So please keep your eyes out for opportunities like that - your voice really does matter.

As to the second question. This is a challenging area in genetics. I just had a conversation with one of our genetic counselors this week about this very question. The best advice is if you have family members who can get tested, this can shed light on your own particular gene mutation. And this information is modified quite a bit. This really points to the importance of our mission, to understand genetic causes of disease. It's scientifically challenging and I'm sure for those who have inconclusive results, it can be very frustrating.

**Jason Menzo, Chief Operating Officer**

Thank you so much, Todd. There's a follow on question about genetic testing that was chatted about intronic gene testing. We can follow up on that question offline.

We had a question chatted in about the CRB1 gene. Rusty, as part of the RD Fund, you have a good handle on the landscape of the portfolio company's and all the stuff happening in research you are familiar with the CRB one landscape maybe you can share an update on that landscape?

**Dr. Rusty Kelley, Senior Vice President, Investments & Alliances:**

CRB1 is an indication that we are paying very close attention to and this primarily comes from the fact that the Foundation has funded work both in Europe and the U.S. (Leiden University and Duke University). The genetics are still being worked out for this disease. There are multiple transcripts involved. I think we are going to make significant progress here shortly given that these research-based studies in academia are moving toward industry. So there is not an actively enrolling clinical trial yet but stay tuned.

**Jason Menzo, Chief Operating Officer**

Thank you, Rusty. Like we always do, we have many questions about specific genes like we had for CRB1. I will use this opportunity to remind everyone at our website, [www.fightingblindness.org](http://www.fightingblindness.org), we have a great amount of resources about the programs happening, preclinical and awards programs that we fund. There's a pipeline of programs that are in clinic or approaching clinic. It's a terrific resource to go to for understanding more about what's happening in the field. We also encourage folks to go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov), which has the listing of any clinical trial happening in our space.

There's a question about if there's a way to donate specifically to a particular study or a particular trial or a particular gene or sometimes we get questions about a particular researcher. As a nonprofit, we have to be really careful in terms of how we take what are called restricted donations. So we have a set of policies and guidelines that we follow. I would encourage folks if you have a question or if you have an interest in a very specific area of research - it often times happens at the gene level - to send a note to [info@fightingblindness.org](mailto:info@fightingblindness.org). The restrictions that we put in place around the level of giving for a gene specific or gene restricted donation is quite large is \$300,000 or greater. If it is something of interest and you have the desire to learn more, I encourage you to reach out and we can have a conversation.

The next question is directed to Amy. There's a question about achromatopsia. The question is based on AGTC and their success with achromatopsia. Is this a genetic editing program or a therapeutic?

**Dr. Amy Laster, Vice President, Science and Awards Programs**

AGTC is a gene therapy company. Their strategy for achromatopsia - two forms of it, the A3 and B3 - are gene therapy strategies.

**Jason Menzo, Chief Operating Officer**

Thank you very much, Dr. Laster. We have about 8 minutes left, so I will make sure that we try to get in as many questions as we can. I will remind everyone any time you have a question that's been chatted into the Insights Forum or asked on Facebook or email or raised your hand or any of the ways that we take questions, if we don't get to it, we follow up with everyone offline.

There's a question that comes up from time to time. How do I know what form of RP I have? This is a great reminder to the importance of genetic testing and our genetic testing program. Todd, can you quickly review how that genetic testing program works for people that have not been genetically tested and want to understand what their gene is?

**Dr. Todd Durham, Vice President, Clinical & Outcomes Research**

For the last year, we have had an open access genetic testing program that comes at no cost to you. There's information on our website and the website of Blueprint Genetics about this program. You will need to approach a health care practitioner who can order a diagnostic test for you. They will order the test through the Blueprint ordering portal, which is called Nucleus. Then Blueprint will

send a test kit to you to collect a DNA sample. Typically, that's saliva. You will send that off and you will be counseled by the genetic counselor once the results return. Hopefully that result would be clear and conclusive for you. The genetic counselor and your health care practitioner will give you guidance from that point. That testing program is available to you at no cost.

**Jason Menzo, Chief Operating Officer**

Thank you very much, Todd. Ben, I am going to direct these next couple of questions to you. There are a number of common questions that we get about clinical development. What percentage of trials that come out of the translational phase actually make it into an IND and Phase 1 clinical testing? What's the time frame from the time that a program goes from Phase 1 testing all the way through to an approved therapy? Setting expectations of all this great work we are hearing about preclinical - what does the road map look like from the point they actually end up in the market?

**Dr. Ben Yerxa, Chief Executive Officer:**

In our space, if you have a preclinical candidate that looks like it's ready for development, it typically takes 18 to 24 months to get through the pre-IND work and get something ready to do the first human testing. From there, it can take anywhere from 6 to 10 years to get to final approval, even if these trials are small. It does take time to recruit, and the FDA requires long-term safety follow up and lots of other stuff that's required for final approval.

The probability of success and failure rates are a little harder to get a handle on. I think that when you are at the preclinical stage, typically your success is about 10%. It goes up dramatically as you get into the clinic and get clinical data from there. Even if you have positive Phase 3 results, your probability of being approved is still probably 90% or so. You never know what the FDA is going to do. You have to keep all of these things in mind. And what we do at the Foundation is try to de-risk program and optimize their probability of success and to speed up their trajectory in the way they get tested.

There's a recent publication that reviewed all gene therapy programs in the clinic and showed that in ophthalmology, as a therapeutic area, had one of the highest probabilities of success in moving from one stage to another. That's actually good for us.

**Peter Ginsberg, EVP, Corporate Development and Chief Business Officer:**

That's right, Ben. If you would like to review the study in full, it's *Nature Reviews Drug Discovery* is the name of the publication and this was published this week January 25, 2021. This study showed is a review of all the clinical studies and they showed that the likelihood of success for a therapeutic gene therapy that just entered the clinic was 31% in the ophthalmology field. Which is pretty good and higher than you would expect for a standard treatment entering clinical trials. As Ben noted, the range of getting into human clinical trials to reaching the market can take 3 years or ten plus years on the long end. It's difficult to assess the timing of early-stage development.

**Jason Menzo, Chief Operating Officer**

Thank you, Peter and Ben. We have time for one more question. The question is about a company called Iveric Bio and any updates on the Best disease program that Iveric had licensed a couple of years ago. And if there's anything else that we can share specific to Best disease.

**Peter Ginsberg, EVP, Corporate Development and Chief Business Officer:**

This is from Iveric's website. They do plan to initiate a study in this setting in the second half of 2021. This will be a Phase 1/2a clinical trial, so the earliest stage of clinical trials. It will be likely a very small study, but we look forward to that study starting and moving forward. As far as I am aware, it would be the first gene therapy into human trials for this indication.

**Jason Menzo, Chief Operating Officer**

Thank you very much Peter. It is just about 2:00 pm here on the East coast, so we are going to wrap things up. As I mentioned a little bit ago, we always capture all the questions that are asked via Facebook, email or hands raised, chat and Q&A in the Zoom interface. Our team follows up with every single question that we didn't get to live. So be on the lookout if you did ask a question that we didn't get to - we will follow up with you in the weeks ahead. I want to take the opportunity to thank everyone for participating in today's call and remind you that there will be a transcript and audio recording of the call within the next week. If there's any information you need or any questions we can answer, our website [www.fightingblindness.org](http://www.fightingblindness.org) is a great resource and of course you can always reach out to us directly by sending us an email at [info@fightingblindness.org](mailto:info@fightingblindness.org). Thank you and have a great rest of your day.