Foundation Fighting Blindness
***Insights Forum* Transcript**
October 29, 2020

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.

I would like to briefly review some 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 in an audio format.

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. 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'm the Chief Operating Officer here at the Foundation Fighting Blindness.

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

We have a very full agenda today.

- First, I will highlight some of our activities and events focused on increasing community engagement, fundraising and public awareness of our mission.
- I will also provide a summary of our audited fiscal year 2020 financial results and a performance update on our first quarter of 2021.
- Then, our CEO, Dr. Ben Yerxa, will share some recent progress and exciting developments related to our research funding.
- We will then be joined by our featured guest speaker, Halden Connor, Chairman, CEO and Co-Founder of Nacuity, a clinical stage pharmaceutical company developing a breakthrough treatment for retinitis pigmentosa and potentially other ophthalmic indications.

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 your 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 accessibility or other suggestions for this call, or the Foundation in general, please reach out to us at the email address: info@FightingBlindness.org.

I want to start today's call by sharing some exciting news. As you may know, the Foundation was founded in September of 1971, and in the 49 years that have followed, many of the critical advancements in our field are directly as a result of our work, including the first ever gene therapy approved to treat certain blinding conditions, and the more than 40 clinical trials that are ongoing today.

There is meaningful progress happening all over the world, and as we prepare to celebrate 50 years of impact, we are launching a new theme for the Foundation called "Together, We're Winning."

This campaign emphasizes all the 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. That said, it's not just about fighting these diseases; it's about celebrating the wins and many small victories along the way.

The "Together, We're Winning" campaign was launched at the beginning of October to coincide with Blindness Awareness Month. As we prepare for our 50th anniversary year

coming up in 2021, expect to see more emphasis on the many ways that we are winning, together as a community.

Building off this campaign, one of our key initiatives in the coming year is to connect more deeply into our communities across the country through our National Chapter Network.

We thoroughly believe that a high performing and well-structured community-based grassroots organization will provide an immense amount of energy and support for our organization. Our vision is to develop an even stronger national chapter network, building on the success of our current footprint and growing it – both in terms of the number of active chapters, as well as the number of engaged members in each.

We have begun executing on our plans with the hiring of an experienced National Director of Chapter Engagement, Ms. Renee Paulsell, who joined our team this week. She joined our team on Monday.

We are very pleased to have Renee on board and look forward to communicating more about our Chapter Development plans in the coming months.

Shifting gears, as we continue to navigate the challenging environment caused by the global pandemic, we've been working to not just navigate, but actually thrive in this new environment.

One of our latest initiatives along these lines is a new series we launched over the summer called "Music to our Eyes". I'm sure many of us on the call here participated in this first event.

"Music to our Eyes" is a livestream music series which features a concert and conversation with a special guest that has a connection to our mission. The purpose of this series is to raise awareness and funds, especially among new audiences who may not be familiar with the Foundation.

Our first event was in August with acclaimed rock band, X Ambassadors. The band shared personal stories of their connection to our mission, and played a few of their top hits including the songs "Renegades" and "Unsteady" and had more than 40,000 views on Facebook. A replay of the event can be found on our YouTube and Facebook pages.

I do want to take a moment to thank our partners on this event - Two Blind Brothers. Two Blind Brothers was founded by Bradford and Bryan Manning, who formed the

apparel company to create ultra-soft clothing, build community, and donate 100 percent of profits towards research to find cures for blinding conditions. We are grateful for their support and for all the participants and donors to this program. The good news, as I mentioned, is that this is a series event that we plan on repeating every quarter with a different act and we're very excited to be announcing the next act in the series in the next couple of weeks – so stay tuned!

Another special day for the Foundation was our Fall National Virtual VisionWalk that was held this past Saturday, October 24th. This one-day event was an opportunity for VisionWalk supporters from across the nation to come together virtually to raise awareness and funds for our mission.

This year, VisionWalkers were encouraged to be creative in how they participated with some walking on treadmills, others going on socially distanced walks at local parks or around their neighborhood, or hosting a virtual meeting with team members.

The program featured teams from across the country meeting and sharing experiences and stories online and was a great success. The event exceeded $1 million raised and adds to the great success of the VisionWalk series. Over the past 15 years, through the generosity of more than 200,000 walkers, donors, and corporate sponsors, VisionWalk has raised more than $55 million to fund research, which is awesome!

Thank you to all who participated. I wanted to share the great news with everyone here today.

In addition to key fundraisers such at the VisionWalk, we also work to partner with corporate and industry players who provide financial support for Foundation initiatives. I'm pleased to announce that we have two new corporate sponsors of My Retina Tracker Program, which offers open access, no-cost genetic testing and genetic counseling for individuals living in the U.S. with a clinical diagnosis of an inherited retinal disease. These corporate partnerships are critical to our success. We look to add more in the weeks and months ahead.

In this current world of virtual events, we continue to search for innovative opportunities for our community to connect.

One of these opportunities is our upcoming National Chapter Webinar, which will be held on Saturday, November 21, 2020, from 12:00 to 1:30 pm ET.

This event is presented by the Foundation Fighting Blindness Arizona and Cincinnati/Northern Kentucky Chapters, and will feature two speakers. Ben

Shaberman, who is the Foundation's Senior Director of Scientific Outreach will provide an update on the latest in research and Foundation news. Dr. Paul Bernstein, a well-recognized expert in nutrition for retinal health, who is with Moran Eye Center at the University of Utah, will present perspectives on nutritional habits and therapies for people with inherited retinal diseases and age-related macular generation.

Another exciting event is planned for Sunday, February 28, 2021 called, "Hope from Home: A United Night to Save Sight".

This event will celebrate Rare Disease Day and help kick off the Foundation's 50th Anniversary year. This interactive virtual experience will feature incredible entertainment and provide an opportunity to connect with others from the comfort of your own home — all while raising critical funds to further our mission.

Before we shift to discuss our financials, I want to highlight a few new items on our website.

The first is a great resource called the "Clinical Trial Pipeline". This resource can be found by going to FightingBlindness.org, then clicking on the Research tab, and then the section called "Clinical Trial Pipeline."

There you will find a list of all known clinical trials being conducted in the inherited retinal disease space, along with their disease focus, phase of progress in clinical development, and the sponsoring organization.

While on our website, you can also check out the official Foundation Fighting Blindness Merchandise Store which we just launched this week! Show off your support of the Foundation with a variety of t-shirts, hats, coffee mugs, and even a few items for your furry friends. These products are an easy way to help increase awareness for the Foundation and support our mission just in time for the holidays.

I'd like to wrap up my section of today's Forum by providing a brief summary of our financial position. As a reminder, the Foundation operates on a Fiscal year that goes from July to June, so our 2020 fiscal year ended on June 30, 2020.

First, our final audited financial results for Fiscal 2020. Our total revenue for fiscal 2020 was $34.7 million, which included $3.1 million of investment income and a new $15 million RD Fund commitment. This was against operating expenses of $14 million. The net result was an organizational surplus of about $6 million which will support new research to add to our previous commitments. The audited financial statements will be

available later this fall on our website in the About Us section under Financial Reporting.

For Fiscal Year 2021, our budget includes targeted revenue of $21.2 million against operating expenses of $14.5 million, with funds to support new research of approximately $6.7 million.

Through the first quarter, we have generated revenue of $2 million against expenses of $3 million which is roughly in-line with our expectations. It's important to note that the first quarter of each fiscal year is typically our lowest revenue level of the year, as fundraising activities gear up. That said, we are experiencing declines in revenue due to COVID-19 and are working to address this through the many creative events and initiatives I just outlined.

We are humbled and grateful by all the support in funding and volunteering that enables us to fund critical research during this challenging time. While we can't predict the short or long-term consequences of this pandemic, we can count on the strong support of our community and can't thank you enough.

I'm now pleased to turn the call over to our CEO, Dr. Ben Yerxa.

Ben Yerxa, Chief Executive Officer

Thank you, Jason. Good afternoon and thank you for joining us on our quarterly update call.

This afternoon, I'd like to provide updates in several areas, including our ongoing core research awards, recent scientific events and the RD Fund. Then I'll introduce our featured guest speaker, Nacuity CEO, Halden Connor.

Let's start with our core research awards.

- We continue to make excellent progress in implementing our five-year Strategic Plan initiatives.
- In August of this year, we announced and designated $6.5 million in funding for 15 new grants, bringing our current research portfolio to a total of 84 grants. The projects were selected from 134 proposal submissions that were rigorously evaluated and scored by the Foundation's Scientific Advisory Board, which is comprised of the world's top retinal disease experts. We just hosted our annual Advisory Board meeting virtually – it's always inspiring to interact with these

physicians and researchers who are leading the efforts to increase our understanding of inherited retinal diseases.

- We are committed to projects that will lead to vision-saving treatments and cures. Our funding strategy also focuses on critical research gaps, that when addressed, will move the whole field forward in a significant way. One key focus over the next few months is our annual call for applications for research project funding.
- We are currently accepting applications for our new translational research acceleration awards. We call these our TRAP awards, which have an important focus on research with particular promise that we would like to accelerate towards clinical trials. The TRAP funding provides a really important bridge between traditional early academic research and the later-stage clinical development funded through the RD Fund. These awards are expected to be made by the end of calendar year 2020.
- We are currently accepting applications for the Free Family AMD Program, which supports research into age-related macular degeneration. These awards are expected to be made by the end of calendar year 2020.
- For other broad ranging research, we are currently accepting applications for Individual Investigator, Clinical Innovation, Career Development & Program Project awards, with Scientific Advisory Board recommendations expected by late spring 2021.

Moving on, I'd like to highlight two recent events held in conjunction with the IRD scientific and medical community.

- In mid-October, we hosted our second online continuing medical education course, which was led by Dr. Rachel Huckfeldt, Associate Surgeon and Director of IRD fellowships at Massachusetts Eye and Ear and Assistant Professor of Ophthalmology at Harvard Medical School. The course covered topics related to care and management of patients with IRDs.
- In mid-September, we also hosted a virtual scientific Workshop on the topic of "Inflammation in Viral Gene Therapy of the Retina".
- The purpose of this event was to bring academia and industry together to present their preclinical and clinical data and openly discuss the observations, challenges and implications of inflammation in retinal gene therapy. The goal was to identify critical knowledge gaps the Foundation may be able to direct research funding towards in the future.
- As way of background, we know that injecting a gene therapy into the eye leads to inflammation in most instances. The amount of inflammation is variable and depends on the specific characteristics of each given gene therapy. Just to be

clear, this inflammation can be controlled clinically, but if we understand the cause of the inflammation, we may be able to help clinicians and researchers improve these therapies even more. We had 25 participants from 5 countries who participated in the workshop and our team is now preparing a summary publication to share the findings publicly.

I'd like to wrap up my remarks today by highlighting some exciting developments related to the RD Fund and our portfolio companies.

- The RD Fund is part of our long-term strategy for adapting to a rapidly changing environment where many more projects are ready for translation into human trials, while the cost of clinical research is increasing. The RD Fund currently has more than $70 million in funding for companies with projects that can be in clinical testing in 18 to 24 months.
- To date, the RD Fund has committed funding to ten companies, including disclosed investments in Atsena, CheckedUp, Nacuity, Nayan, ProQR, SparingVision, Stargazer, and Vedere Bio.

There have been several exciting recent developments that I'd like to summarize.

SparingVision is a French biotechnology company developing a gene-independent treatment for retinitis pigmentosa, the most common inherited retinal degeneration. There is currently no treatment to treat all genetic forms of this rare retinal disease that leads to blindness and affects nearly 2 million people worldwide.

- Last week, SparingVision made several important strategic announcements.
- It was announced that the Company's Chairman of the Board, Stéphane Boissel, has been appointed Chief Executive Officer of SparingVision.
- And the Company announced that it has raised new funding through a 44.5 million Euro financing round, which translates to roughly 53 million in U.S. dollars.
- Several new leading global investors participated in the financing, including 4BIO Capital, UPMC Enterprises, Jeito Capital and Ysios Capital. In addition, current investors, the RD Fund and Bpifrance, participated in the financing.
- The RD Fund invested an additional $6 million dollars in this round, which builds on the Fund's original $8 million investment in 2016.
- The proceeds from the financing will be primarily used to advance the development of SparingVision's breakthrough treatment SPVN06 for RP.

- The Company is are preparing to conduct an initial clinical safety study, scheduled to start in 2021. Clinical trials with an endpoint in RP may take 1-2 years of follow up. So the funding helps the Company complete Phase 1 testing, but also provides for undertaking and getting a readout of a subsequent Phase 2 trial.

The other exciting recent development from the RD Fund relates to Vedere Bio, a biotech company that is focused on next generation optogenetic gene therapy as an approach to restore vision in eyes that have lost most vison due to degeneration of photoreceptors.

This is a technology that has the potential to work regardless of the genetic cause of the disease and it works by introducing a light-responsive gene into cells that do not normally respond to light, making them light sensitive.

- The Company's technology is based on work primarily from the labs of Drs. Udie Isacoff and John Flannery of UC Berkeley and technology from the University of Pennsylvania. Vedere Bio, was launched in June 2019 with a $21 million financing led by Atlas Venture, of which the RD Fund contributed $3 million, effectively leveraging its capital 7-fold. Because of the competitive nature of this field, the company remained in "stealth mode" until this time.
- With $21 million of fresh capital, the new team was able to make amazing progress in just 14 months to generate a viable clinical candidate for optogenetic gene therapy.
- This fast moving, advanced technology caught the attention of industry leaders and now we are pleased to announce that Vedere Bio has been acquired by Novartis for $150 million in upfront payments, and with milestones, the total economics are approximately $280 million.
- This represents the first exit for the RD Fund and validates the power of the venture philanthropy model for accelerating our mission while providing for meaningful returns to support the Foundation's mission. Importantly, this new technology is now in the hands of skilled developers who plan to invest significant resources to bring this technology into the clinic, and if successful, ultimately to patients in need.
- One additional benefit of this transaction is that the RD Fund has also invested in the same team who formed Vedere Bio II, a spinout of Vedere Bio I, which is working on next generation gene therapies for retinal degenerative diseases.

These recent announcements from SparingVision and Vedere Bio are strong proof points of the impact of venture philanthropy in achieving real and accelerated progress in the development of new therapies for patients with vision loss. For the millions of people globally living with inherited retinal degenerations, the advancement of novel therapies provides hope for the future.

Another important company that is developing promising therapies targeted towards a broad range of IRD diseases is Nacuity.

- The Company is working on a breakthrough treatment for retinitis pigmentosa and Usher syndrome addressing oxidative stress in the retina, which causes cell degeneration and vision loss in virtually all forms of RP. We are hopeful that Nacuity's approach using N-acetylcysteine-amide (NACA), with its anti-oxidative properties, can benefit most people with RP, regardless of the gene mutation causing their disease.
- We are excited the NACA drug has completed Phase I clinical trials and is proceeding to Phase 2.
- The Foundation, through our RD Fund, has committed to providing initial milestone-based and equity funding of $7.5 million to advance the potential therapy into and through a Phase 2 clinical trial.
- We are pleased to have on the call with us today, Halden Conner, Nacuity's Chairman, CEO, and Co-Founder, to share an update on their programs and clinical progress. Halden helped co-found Nacuity in 2016. During his extensive career in the ophthalmology field, he has served as a Board Director for Alcon Laboratories and as an Advisory Council Member for Wilmer Eye Institute. He was also the co-founder of ProTom International, a leading device manufacturer of proton therapy technology.

Without further ado, I'd like to welcome Halden and turn the call over to him. Halden, please go ahead.

Halden Conner, Chairman, CEO, and Co-Founder, Nacuity

Thank you for the introduction, Ben, and good afternoon or morning everyone, as the case may be. It is a great pleasure to take part in this October edition of the Foundation Fighting Blindness Insights Forum. I want to thank Ben, Jason, Chris and the rest of the Foundation team for the invitation to speak here today. It is always a

pleasure to speak to this community about our work, particularly given how important the Foundation has been to our progress, both financially and through their scientific support.

As Ben said, my name is Halden Conner and I am CEO and co-founder of Nacuity Pharmaceuticals. Nacuity is a clinical stage pharmaceutical company based in Fort Worth, TX with a mission to advance a promising therapy to slow or stop the progression of the blinding effects of retinitis pigmentosa. We follow and build on the research findings that originated in the lab of Dr. Peter Campochiaro at the Wilmer Institute at Johns Hopkins University which also benefited through funding from the Foundation. Through his research and clinical observations Dr. Campochiaro concluded that in patients with RP, photoreceptors, rods and cones, were differently affected by the multitude of genetic mutations that cause RP. He believed the genetic mutations mostly damaged only the rods, that the death of the rods created significant oxygen overload, or oxidative stress, in the retina, and that oxidative stress in turn compromised the cones, leading to loss of central vision.

About that time, I was involved with a group exploring neurological and other uses of the small molecule antioxidant N-AcetylCysteine Amide, or NACA and that group believed it could be useful in ophthalmological conditions. Accordingly, I reached out to Dr. Campochiaro, learned of his work exploring antioxidants for RP and asked if he would be interested in evaluating NACA, which he was. Once we provided him the NACA he studied the effects of orally administered NACA in a mouse model of RP, "rd10 mice," with very favorable results. Those results, along with confirming belief from other members of the ophthalmological drug development community, inspired us to found Nacuity to advance this promising therapy for RP.

Fortunately, we are based in Fort Worth, TX, the home of Alcon Laboratories, one of the leading ophthalmological pharmaceutical companies in the world. As I have a longstanding relationship with Alcon and many of its key leaders and scientists, I was able to attract some top talent with years of productive experience at Alcon to join Nacuity, which has proven to be an invaluable asset as we have proceeded forward.

Our team has been successful in producing a Good Manufacturing Practices grade of NACA we designated NPI-001, putting it in tablet form for easy administration, completing the required pre-clinical toxicology programs, successfully filing an IND with FDA, completing a Phase I Clinical trial in healthy volunteers in Australia, and gaining ethics committee approval for and beginning a Phase I/II trial, also in Australia, focused on the treatment of RP in patients with Usher Syndrome. We are very excited that our RP trial for Usher has thus far enrolled four patients who have been on drug for thirty

days, and after a planned safety assessment in November, the study will open for full enrollment.

We expect to have our first round of safety data from this trial by the fourth quarter of next year, and findings of efficacy by mid to late 2022.

As Usher Syndrome is an ultra-orphan disease, and if our findings of safety and efficacy warranted such action, we would approach the Therapeutic Drugs Administration in Australia, the equivalent of the U.S. FDA, seeking expanded use for NPI-001, thus providing the earliest access to the drug for Usher and RP patients. Assuming our safety data is positive in late 2021, we plan to amend our U.S. IND with the FDA in preparation for a pivotal Phase 2 trial in the U.S. for patients with RP.

As we have sought other opportunities for ophthalmic use of our expanding library of small antioxidant molecules, the Foundation suggested we explore use of our antioxidants for cataract prevention since they were aware RP patients develop cataracts to a greater extent than the general population, likely due to the elevated oxidative stress in the eye due to death of the rods. Therefore, we began exploring use of our compounds for slowing the progression of cataracts by delivering our antioxidants via a biodegradable intravitreal implant. We have demonstrated that our drug elutes favorably from the implant after insertion into the vitreous humor, and we are presently conducting a nonclinical safety study of our antioxidant infused implant.

We have been extremely fortunate to have an outstanding Scientific Advisory Board guiding us over the past several years and for the purpose of our anticataract product development, we were most pleased to have recently added to our team, at the suggestion of Ben Yerxa, Dr. Nancy Holekamp, Professor of Clinical Ophthalmology and Visual Sciences at the Washington University School of Medicine in St. Louis, Missouri. Dr. Holekamp is a pioneering researcher, and leading authority, on the role of oxidative stress in the formation of cataracts and is enthusiastically advising us about this program. Assuming our toxicology study results are favorable, we plan to initiate a proof of concept clinical study early next year and expect to have preliminary results by the end of 2021.

The ability of our compounds to attenuate the effects of oxidative stress has the Nacuity team looking at the effects of our compounds in other ophthalmological conditions. Thus we continue to actively synthesize and screen our compounds for efficacy in these other ophthalmic diseases involving oxidative stress. Our entire team is very excited to lead the way in exploring treatments for ophthalmological diseases

involving oxidative stress and we are very pleased to be in the clinic to demonstrate the value of NPI-001 for the treatment of RP.

Your participation in this Insights Forum is appreciated, as is the opportunity to update you on Nacuity's progress. I want to reiterate our thanks to the Foundation Fighting Blindness for their continued support of our work without which our hope of delivering meaningful assistance to the IRD community would be sorely compromised.

I appreciate your time and attention. I'd now like to turn the call back over to Jason.

Jason Menzo, Chief Operating Officer

Thank you, Halden, for that informative update. It's exciting to consider a broad potential treatment that could address the needs of the nearly 2 million people who are afflicted with RP worldwide. We're all watching and anxious to hear more progress in the months ahead. Much success to you.

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:

As a reminder 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.

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

While we are compiling questions, I'd like to take a moment to thank everyone for participating today and remind you that there will be a transcript and audio recording of today's call on our website within the next week. If there is any other information you need, please reach out to us by sending an email to info@fightingblindness.org. We're also streaming now live on Facebook. Hi, everyone on Facebook! For those who are chatting on Facebook, our team is monitoring the questions that come up there as well and we'll bring them into the discussion here as appropriate.

Let's take our first question about the pandemic is impacting retail rather than in terms of federal funding, timelines and really, just the general state of affairs in the clinical world, as it relates to the pandemic. I can direct that first question about the impact of the pandemic to Dr. Brian Mansfield, who is our Interim Chief Scientific Officer.

Dr. Brian Mansfield, EVP of Research, and Interim Chief Scientific Officer

Thank you, Jason. I think this is the question that we really don't know the answer to yet. Clear, the pandemic is not over. We've been through a very chronic, short hard phase for a while, where there were a lot of close-downs, across states but of course, some states remained open and the response to research labs for the COVID crisis vary from state to state, from institution to institution and now, of course, we have seen resurgence of COVID in other states and again, this is going to be leading to consequences in those states. So until the pandemic is over, we really don't know the full impact of what's going on. So let me share with you what we do know about what has been happening in nearly all states, whether they closed down or not. Critical long-term oriented research was allowed to continue. There was access for a limited number of people into labs to ensure that those critical long-time length experiments were allowed to go on. A lot academic institutions opened up slowly since the burst of COVID, and they returned to half or a third of the people. People were cycled in and out, to try to bring their experiments back up and to keep them flowing.

So there's not been a complete close-down of all of that research, moreover, a lot of people of course using that time, even though they may have not been able to go into the lab, to do other work, which is very productive. For instance, they are writing up reports, publications, planning experiments, submitting grants for the next funding round. The NIH doesn't tend to hold those other rounds, just as we have not, for funding ongoing research. So we wait. We monitor. We try to understand the impacts and we will all try to respond to them as best as we can. The NIH has made it very clear it is well aware of these impacts and is monitoring them and intends to be able to assist people to the best of its budgetary ability, as we fully understand the need and the

impact. I hope that helps give you a sense of what is going on at the moment in this current environment.

Jason Menzo, Chief Operating Officer

Very good. I think it was a great question and topical and probably on a lot of folks' minds right now in terms of the pandemic and its impact on our mission and our field. Thank you very much for that, Brian.

We have a number of questions for Halden. Can you speak on the clinical trial you referenced in your presentation? It's happening right now in Australia. I know you mentioned there were four patients currently enrolled and mentioned it's happening in patients that have Usher's Syndrome. One of the questions is:

Can you discuss the subtype of Usher's Syndrome that you are looking to enroll in this trial. And then second, can you speak a little bit more directly to the goal of the trial, be it testing safety, efficacy end points and things of that nature. So, it's combining a couple of questions into one if you don't mind taking that.

Halden Conner, Chairman, CEO, and Co-Founder, Nacuity

It is a double blinded, 48-participant trial. It required a first round of a vanguard group which we completed and then we go to full enrollment. There seems to be a great deal of enthusiasm so we expect good progress in our enrollment into the early part of next year. I will have to tell you that the RP associated with usher, while we picked Usher because it has a potential for a fast track, as an ultra orphan, the manifestations of RP are exactly the same as in any strain of RP, so what we learn should be applicable across all forms and subsets of RP. Borrowing a little bit from Brian, I would mention that it's fortuitous that we decided to implement this trial in Australia where we designed our initial safety study because Australia has had far less impact from COVID-19 than we have had here in this country, and other than some minor delays, we don't anticipate any slowdowns in the trial due to COVID-19.

Jason Menzo, Chief Operating Officer

Excellent. One follow-up question. When the trial is beginning enrollment, eventually perhaps here in the United States, how would an individual know to be considered for enrollment in the clinical trials?

Halden Conner, Chairman, CEO, and Co-Founder, Nacuity

Once we have a cleared IND to proceed with a full Phase 2 trial in the U.S., I'm sure the Foundation will be helpful in spreading that news, as we will, and obviously, anybody in My Retina Tracker or any other association with RP, with the Foundation is going to know about it and be alerted to possible participation.

Jason Menzo, Chief Operating Officer

All Usher Syndrome patients can participate, not a subset, as long as they meet the RP criteria, is that correct?

Halden Conner, Chairman, CEO, and Co-Founder, Nacuity

That is correct.

Jason Menzo, Chief Operating Officer

Thank you very much. We have a number of questions I'm going to combine into one. Really, they are related to the patient experience with RP. One patient said it looks like a fog, other days it's clear. Another patient saying that their progression is very different in one eye as compared to another. And obviously, every patient may experience the clinical signs of RP differently. But Brian, maybe I can ask you to speak to some of the more traditional patient journeys, related to clinical signs and symptoms of RP, to try to answer some of those questions.

Dr. Brian Mansfield, EVP of Research, and Interim Chief Scientific Officer

Thank you, Jason. The patient reported outcomes and experiences are an area of great interest in inherited retail diseases. Obviously, if you start to develop a therapeutic, you need to know what people would like to have resolved in the disease. Often the experience involves more than just a concern about visual acuity, as some of these questions are indicating. And to get to understand that, industry now does start holding what they call patient focus and patient journey groups, where either before developing therapy or in the early stages of developing a therapy they seek people who are in the group of patients that they would like to develop the therapy for and hold discussions with them about these questions, trying to understand what is the experience of patients with these conditions and what would they like changed about those conditions.

The registry, My Retina Tracker, receives almost weekly requests now, from various industry partners to help find groups that can range anywhere from 2 to 20 people to, take part in industry-run focus groups and patient journey groups. So, this is

something that is a strong interest and in addition, the Foundation realizes that is this is very important and understanding whether these symptoms and occurrence resolve throughout course of the therapy, is also important to capture. And mostly, these are not characteristics called by the normal clinical measurements, for instance, of your peripheral vision or vision acuity. To that extent, the Foundation is partnering to develop what we call PRO or patient-reported outcomes and under the guidance of Dr. Todd Durham, our Vice President of Clinical Outcomes research, we are developing surveys which try to collect exactly this sort of information, documented and analyze it scientifically to give a usable end point to therapies, as they are developed and tested.

Jason Menzo, Chief Operating Officer

Thank you, very much, Brian. I know we have an individual who has raised their hand.

Participant

Thank you very much. My question on the Nacuity intervention has to do with stage of disease. So it sounds to me that this particular intervention is probably targeted more to individuals who have some threshold of remaining vision, remaining viable cone cells, versus those who have late stage disease with few remaining photo receptor cells to work with and therefore, I would assume the participants in the trial at this point are probably individuals with earlier stage disease and later stage disease. I just wonder if you would comment on that.

Halden Conner, Chairman, CEO, and Co-Founder, Nacuity

Yes. Since the death of the rods occurs first, and peripheral and night vision starts to fade, for the trial, ideally, we're look for people that still have good cones and central vision and see if we can slow the progression of that blinding effect. So you are correct. Once the cones are gone, there's nothing we can do to retrieve them. We're just trying to lower the oxidative stress to the degree that the cones survive longer.

Jason Menzo, Chief Operating Officer

Ben, I'm going to direct a question to you about the RD Fund. There were several updates mentioned in the call earlier today on the RD Fund. If there's any additional information you could share regarding the announcement this morning from Vedere Bio. From a science perspective, in terms of the target and the cell type in particular, but also, just generally, now that we have some success, what do you envision, going forward in terms of the strategy and some of the things we could expect that from the RD Fund, going forward.

Ben Yerxa, Chief Executive Officer

Regarding the first question, on the Vedere Bio technology, we're not at liberty to disclose any more scientific information from the Company, but the base technology that was licensed by the Company was published by John Flannery in two fairly recent publications. If anyone has trouble finding those, we can get them to you as well. But they should be pretty easy to find. That goes into the science into very great detail. Regarding the RD Fund strategy, we are fairly late in that Fund. We have got 10 investments. What we're trying to do is basically cover a lot of different modalities. We want to cover as much of the IRD space as possible in terms of gene agnostic technologies, a few gene-specifics here and there, but really to cover early mid and late-stage disease with a variety of different approaches. So it's a bit of a challenge to do that but i think we're fairly well-balanced. I'd say that one of the missing things that we're looking seriously at is regenerative medicine. We haven't found an investment in that space that meets our criteria yet, but we're actively looking.

Jason Menzo, Chief Operating Officer

Thank you Ben. Brian, I'm going to address a question to you on genetic testing. Number one, can you remind folks about how they can get involved in our genetic testing program and My Retina Tracker. And number two, there are a number of folks that have stated, they had genetic testing done and either there was not a conclusive result that was found in the genetic testing or it's been some time since they were genetical tested, and they are curious if they should get tested again. And finally, if it was recently they were genetically tested but there was not a conclusive result, what is the most important thing for someone with RP to do if there's not a clear genetic result.

Dr. Brian Mansfield, EVP of Research, and Interim Chief Scientific Officer

Let me start out by explaining the current situation for getting a genetic test. The Foundation has a genetic testing program, associated with its My Retina Tracker registry. You do not need to be a member of the registry to order the test. We'd love for to you join, but you don't have to be a member. What you need to do is approach the clinician, who is looking after your eye care and ask them if they are able to order the genetic test from a company called Blueprint Genetics. Now, it can only be ordered by a clinician. So unfortunately, i and others in the Foundation are not able to order it for you. You are not able to order it for yourself. You need to ask your clinician to do it. If the clinician is not aware of the program or doesn't know how to get in touch with Blueprint Genetics, you can either ask them to contact us at the Foundation or more

easily, go to the Foundation's web page and type in My Retina Tracker genetic testing. It will take you to a page where there will be information about the testing program that will be in a pdf document that you can download for yourself, describing what genetic testing is and what it will tell you and what it won't tell you. And there will also be a document there that can you download for your clinician or the clinician can download for himself and it tells the clinician how to go about ordering the test.

The test is one of the most comprehensive genetic test for inherited retinal diseases available commercially. The doctor, again, needs to order it for you. No one else can do it. It has to come from a clinician. The test itself, when the sample is received, and the sample could be saliva or blood, it's your clinician's choice which one, takes somewhere between 4 to 6 weeks to get results which will be given to a genetic counselor. That test is totally free. The Foundation has a program through its partnerships, which will allow us to cover the cost of that test. The genetic counseling is also provided free of charge by the Foundation and the genetic counseling is something that's really important to follow up on because it explains to you what the test tells you. And these tests are really complicated to read. They are not like a blood test where you have a high other low, normal or going like that. They are very difficult to read, essentially, they are hieroglyphics of genes and mutations of variants and VUS's and all sorts of things that many of us don't talk about. But a genetic counselor puts that into easy to understand lay terms and they'll explain what it means to you and your family, but also tells you about any clinical trials you may be interested in looking into further, and telling you about some of the preclinical research that's going on.

So it's a really informative session. During that counseling if you're not a member of the registry, the counselor may ask you if you'd like to join. If you are, they will help you, there and then online to create a profile in the registry.

The eligibility criteria for being tested comes to the other question that Jason asked. At the moment to get the test, you have to have a clinician diagnose you with an inherited retinal disease; the clinician needs to affirm that you have not had a genetic test that looked at more than 32 genes. Now, our test looks at 332. So 10 fold more. You must not have tested for more than 32 genes during or after 2016. Now, the reason is, the number of new genes we have discovered since 2016 has dropped off dramatically and those genes tend to explain only very rare cases of disease. So if you've had a good test before 2016, it's probably a good time to be tested again. If you've had a test since 2016, and not come up with the result, being tested on our panel is very unlikely to help you identify the genetic cause of your disease. In that case you're best to talk with your clinician about what can you do with your current disease - are there any actions

can you take to slow the progression of the disease. They are really in the best position to advise you. I'm not a clinician. I can't give you any medical guidance along those lines.

Those of you who find you cannot discover your gene - you're not alone. About 50% of people who go through testing will not get a clear cause of disease from the test and the Foundation is well aware of this knowledge gap and that is one of the reasons that we fund a multi investigative program, which we call the elusive genes program, whereby we search for the genetic cause of the diseases which currently cannot be diagnosed by the current testing platforms. And they are discovering, one gene at a time, slowly. These are hard genes to find, but they're coming and as they come, they are added to our panels and at some stage, we will change our guidance on when it's best to be tested again, if you've already been tested.

Jason Menzo, Chief Operating Officer

Thank you Brian. I recognize it's 2:00 here on the East coast. We have a couple of good questions. I'm basically going to make one comment and maybe one last question and then we'll wrap the call up. There are a number of questions on what to do, now that you may have a diagnosis for inherited retinal disease.

I mentioned in the earlier part of the call, we do have a National Chapter webinar, that's going to feature discussions around nutrition. The webinar is November 21, from 12:00 to 1:30 eastern time. And the audience is really open to anyone. Even though it's put on by two of our key chapters, anyone can participate and that would be a great place to get additional information about nutrition.

The last question I'm going to ask to you answer, Ben, is about the regulatory environment. There are a number of folks that have asked, now that a gene therapy has been approved, is that an accelerator for gene therapies being approved or how does the regulatory environment look at additional treatment options, using the gene therapy modality, now that you've got one gene therapy approved. And maybe tacking onto that, does the accelerated approvals around COVID-19, play into or impact the regulatory pathway in our space. So, two regulatory questions I'll pose to you and then we'll wrap up the call.

Ben Yerxa, Chief Executive Officer

What the Luxturna program did for the field was provide a roadmap. They essentially blazed a trail and created a playbook for how to develop new therapies. Every program is going to be evaluated on its own merits for safety and efficacy. So they still have to

go through the same process but it's a little bit easy to make decisions about the approach, based on some of the experiences of the previous approval. So in a sense, it makes the field wide open. That's why we have 40 plus clinical trials going on. People understand the road map. But it doesn't necessarily accelerate. It might a little bit in terms of decision making and maybe less mistakes made along the way. But every program still has to go through all the same steps. Regarding COVID-19, it doesn't necessarily help or hurt us. As long as the FDA is staffed well enough to be able to handle all of these new applications coming their way. I think that's fine. You know it's not a national emergency to evaluate the IRDs. They have their priorities based on the pandemic. But the pandemic is going to be under control here at some point, it's not going to last forever. Things will normalize, and the staff and the novel programs coming their wake will be fine.

Jason Menzo, Chief Operating Officer

Thank you, Ben, and thank you to everyone for participating in today's call. It's a few minutes after 2:00 here on the East coast. We're going to wrap things up. For any questions that we weren't able to answer live today on the call, we will follow up with you via e-mail. We do it every time. We always have a long list of questions and our team diligently reaches out to every individual that asked a question personally. So we will reach out to you if we didn't get to your question on the call today. We really do appreciate everyone’s engagement throughout the call and look forward to our next Insights Forum call in a few months. Thank you all very much and have a great rest of your day. Stay safe.