Foundation Fighting Blindness
***Insights Forum* Call Transcript**
October 30, 2019

Chris Adams, VP, Marketing & Communications:

Good afternoon and welcome to the Foundation Fighting Blindness Insights Forum Call. I am Chris Adams, Vice President, Marketing & Communications at the Foundation. We appreciate everyone joining us for today's Forum. I would like to briefly review some logistical details for the call. Currently, all lines are in listen-only mode. 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 today's event does not have any slides. It is purely an audio presentation.

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At this time, I would like to turn the conference over to Jason Menzo.

Jason Menzo, Chief Operating Officer:

Thank you, Chris. Good afternoon. My name is Jason Menzo, and I am the chief operating officer here at the Foundation Fighting Blindness. Thank you for joining us today. I'd like to welcome everyone to our quarterly Insights Forum call. The purpose of these calls is to highlight the latest developments here at the Foundation Fighting Blindness and provide updates on our progress towards our mission: to drive and

accelerate the search for preventions, treatments and cures for inherited retinal diseases.

On today's call our CEO, Dr. Ben Yerxa, will provide a strategic update; I will then provide an operational and financial review; and finally, our Executive Vice President of Research, and Interim Chief Scientific Officer, Dr. Brian Mansfield, will share a research update.

We will have a question-and-answer period at the end of the call, and at that time, Chris will repeat the instructions on how to ask your questions. A replay and fully accessible transcript of this call will be available on our website in the weeks ahead. Also, this call is being closed captioned. If you have any feedback related to accessibility or other suggestions for this call, please reach out to us at info@FightingBlindness.org.

I'd now like to turn the call over to our CEO, Dr. Ben Yerxa.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you, Jason. Good afternoon and thank you for joining us on our quarterly update call for the inherited retinal disease community. The Foundation plays a critical role in the fight to end blindness caused by inherited retinal diseases and macular degeneration.

I'd like to provide an update on recent developments both at the Foundation and within the broader community.

First, let me briefly summarize some of the notable takeaways at the recent American Academy of Ophthalmology (AAO) annual meeting which took place earlier this month. The Academy's conference is one of the premier global eye care medical meetings with more than 15,000 ophthalmologists and health professionals attending from 130 plus countries.

There was an inaugural pre-conference meeting focused on IRDs called "The Future is Now," that the Foundation co-sponsored and participated in. The meeting brought together many leading physicians, biotech and pharma companies, manufacturers and contract research organizations. It's great to see this momentum and increased podium time around the Academy meeting.

At AAO, besides lots of meetings with our key players in the IRD space, there were

some new clinical data announcements:

ProQR announced updated interim clinical data on their CEP290 Ph1/2 clinical trial showing nice durability of response as well as safety data up to 1 year in some patients. Brian will provide more detail in his remarks.

ReNeuron also released late breaking interim data from their Ph2a trial in retinitis pigmentosa or RP patients, with some positive signals of efficacy.

Although not announced at the Academy meeting, it is still worth mentioning here that iVERIC Bio announced this week positive Ph2b clinical trial results of Zimura for the treatment of geographic atrophy associated with age related macular degeneration. iVERIC is also testing the same drug for Stargardt disease, with interim results expected in mid to late 2020.

As with many rare disease clinical trials, the early Ph2 data often deal with small numbers and the results can be somewhat difficult to interpret and extrapolate until the larger and longer trials are completed, but these are steps in the right direction. We will continue to monitor and provide updates.

Next, I'd like to provide an update on our Retinal Degeneration Fund. Launched in late 2018, 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 initial funding to invest in companies with projects that can be in clinical testing in 18 to 24 months.

To date, the RD Fund has committed funding to six companies – Nacuity, Nayan Therapeutics, ProQR, SparingVision, Limelight Bio and Vedere Bio – totaling $42 million in currently committed capital and reserves. It is truly inspiring to see the innovation these companies are developing. I'll give you a quick overview on these companies.

Nacuity is a clinical stage pharmaceutical company whose mission is to develop a breakthrough treatment for RP. Their lead technology is based on studies from the laboratory of Dr. Peter Campochiaro at the Wilmer Eye Institute involving oxidative stress in the retina. Nacuity continues to make progress towards its planned Phase 2 clinical study in RP patients.

Nayan Therapeutics is a preclinical stage company developing mutation-agnostic therapies to treat inherited retinal diseases. The company was founded in February 2019 based on research from Dr. Tom Reh's lab at the University of Washington. Dr. Reh's research has been partially funded by a Fighting Blindness Translational Research

Acceleration Program Award. 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.

ProQR is a clinical state company developing transformative RNA medicines for the treatment of severe genetic rare diseases such as Leber's congenital amaurosis 10, Usher syndrome type 2 and autosomal dominant RP. ProQR's program for Ush2A that the Foundation is co-funding is currently enrolling patients in its Phase 2a clinical trial.

SparingVision, a French biotechnology company developing therapies for retinal degenerative diseases such as RP, is developing an emerging, cross-cutting gene therapy designed to preserve cone photoreceptors, thereby saving vision, in people with many forms of RP. The company plans to initiate a clinical trial for the treatment in the US and Europe in late 2020.

I'm pleased to report that we have two new additions to our RD Fund portfolio:

Limelight Bio is emerging biotech company developing novel gene therapies to enable the treatment of debilitating inherited diseases, including those that cannot be addressed by current technologies; such as IRD's caused by mutations in large genes and autosomal dominant inheritance patterns. Limelight was founded by experienced scientific leaders from the University of Pennsylvania. At the beginning of this month, Dr. Michael Ehlers was appointed CEO of Limelight Bio. Most recently, he was Executive Vice President and Head of R&D at Biogen.

The other recent RD Fund investment is with Vedere Bio, a biotech company that is focused on next generation optogenetic gene therapy as a pan-retinal approach to restore vision in eyes with little to no remaining photoreceptors. Spun out of the University of California, Berkeley, Vedere is developing cutting-edge gene therapy products to restore functional vision to patients who have suffered vision loss from IRDs, as well as other causes of both genetic and non-genetic vision loss. While the vast majority of ocular gene therapies only limit the rate of inevitable vision loss, Vedere aims to restore lost vision regardless of a patient's underlying genetics or their stage of disease.

In summary, we are very pleased that the RD Fund is generating significant interest, with many exciting and worthy investment opportunities coming forth regularly. You can find more information about the fund at RetinalDegenerationFund.org.

Moving on, I'm pleased to announce that we have several new members of our Scientific Advisory Board. Our highly experienced Advisory Board is comprised of

approximately 50 of the world's leading retinal experts who provide insight to the Foundation on research and clinical advancements and review research grant applications. The key opinion leaders in our field volunteer their time to make a difference in the IRD community. We are grateful for the service and commitment of our existing members and are pleased to welcome four new members including:

- Dr. Jeffery Boatright from Emory University
- Dr. Jason Comander from Massachusetts Eye and Ear
- Dr. Thiran Jayasundera from the University of Michigan; and
- Dr. Goldis Malek from Duke University.

To conclude my update, I'd like to provide a quick story about how working together benefits our community and advances critical research. I recently attended the Sophia Sees Hope Family Conference and was touched by a hug that occurred from across the room. One of the attendees was a volunteer in the early Luxturna clinical trials as was in fact the first person to receive bilateral gene therapy injections in her eyes, both eyes. This was a big deal at the time and an important technical and safety hurdle that needed to be overcome in order to progress the therapy. She was greeted by a mom, whose child was diagnosed with RPE65 LCA in January of this year, who then received bilateral treatment with Luxturna in June, and now can see fruit flies in the kitchen from across the room. It was a big hug and a thank you… We at the Foundation want to thank everyone in the IRD community for everything you are doing to help prevent and treat these blinding diseases.

I'd now like to turn the call over to Jason Menzo, our Chief Operating Officer. Jason …

Jason Menzo, Chief Operating Officer:

Thank you, Ben. I'd now like to provide an update on some of our key operational initiatives.

An area of emphasis over the past few years has been on improving our data, systems and processes to drive more automation and efficiency into our operations. In that time, we have made multiple system and structural enhancements to the backbone infrastructure of our organization. Many of these enhancements have been behind the scenes, while others are more visible to our broader community, such as our new online fundraising platform, Classy, which is used for our VisionWalks.

These improvements help provide more timely performance reporting for our organization, reduce administrative burdens and enable our staff to focus on research, outreach and advocacy, and community engagement efforts.

While we have made progress in strengthening our infrastructure, we are committed to continuous improvement and continue to look for opportunities to be more efficient and effective. In all cases, our goal is to improve the experience of our constituents and enable our team to do the important work of driving our mission. [pause]

Another area of recent focus has been our new Community Engagement & Professional Outreach team, which enables us to offer rapid, credible and personal outreach and responses to individuals and families dealing with inherited retinal diseases along with the healthcare professionals working with them.

We have constructed a comprehensive “welcome” experience for newly diagnosed patients when they and their families are first introduced to the Foundation.

We are also working directly with eye care professionals to educate them on the many resources available to their patients with an inherited retinal disease. Our efforts have been well received, with strong interest from many ophthalmologists and optometrists for presentations, brochures, slide decks and other materials to share with their patients.

One great example of a recent connection is with the University of Alabama at Birmingham School of Optometry, one of the largest and most respected schools of Optometry in the country.

Our professional outreach team was invited to share information about the Foundation’s resources and research to the school’s graduating doctors. This was an opportunity to engage optometrists right before they begin their clinical practice, fostering an early relationship between these professionals and the Foundation.

In addition, our outreach program has been working with local Foundation chapter leaders (many of whom are on this call today!) to reach large clinics and physician offices all over the country.

Our Marketing, Communications and Corporate Partnerships teams have also been making great progress in advancing our efforts to raise awareness of the Foundation, increasing engagement throughout our communities, and identifying additional avenues to raise more funding for research.

As part of our efforts to build awareness for the Foundation, we have developed a

brand-new Public Service Announcement (PSA) campaign.

The direction of the campaign is about empowerment and speaks to the idea that no matter what an individual's diagnosis is, it's their drive that can motivate them to follow their passions. With everyone in our community working together, we are stronger, and can accomplish our mission.

We are proud to be featuring several members of the Foundation Fighting Blindness community in the campaign, which has started to roll out across tv, radio and magazine outlets.

Speaking of community, we have two major events planned in 2020 to bring together the many stakeholders who are involved with the Foundation.

First, we will be hosting the Investing in Cures Summit on March 13 & 14, 2020 in Raleigh, North Carolina. This event is focused on bringing together a cross section of industry partners, investors, translational research experts, clinical trial investigators, and the companies poised to take emerging therapies across the finish line. Based on the success of our 2019 meeting, we have decided to host this event annually.

Second, we are looking forward to our VISIONS Conference, which we host every other year bringing together our global IRD community. This conference will be held from June 18-20, 2020 at the Hyatt Regency Minneapolis. We are planning a great event with a comprehensive program of scientific presentations, clinical updates, patient-focused sessions and recognition of community member achievements. We hope you will all join us. Stay tuned for more information as the conference gets closer.

I'd like to wrap up 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 2019 fiscal year ended on June 30, 2019.

In Fiscal 2019, we outperformed our planned budget with revenue and support of $56 million, and operating expenses of $11 million. Of that $56 million in revenue, $47 million of those funds are allocated toward research support, including $24 million of funding dedicated to our recently established RD Fund.

For Fiscal Year 2020, our budget includes targeted revenue of $25.6 million with expenses of $15.3 million, with funds to support new research projects of approximately $8.8 million.

As of September 30, 2019, our year-to-date Fiscal Year 2020 unrestricted revenue was approximately $4.8 million and expenses were $3.4 million which is on track with our

expectations for the quarter.

We are truly grateful for the generous support of all of our donors who continue to support the Foundation each year through our events, annual giving and major gifts. We are deploying these funds as efficiently as possible directly towards cutting-edge research. Now for an update on that science and research landscape, I'd like to turn the call over to Dr. Brian Mansfield, our EVP of Research and Interim Chief Scientific Officer.

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

Thank you very much, Jason. I'm pleased to have the opportunity to provide an update on the many exciting scientific and clinical research developments happening in the area of retinal disease.

This afternoon, I'm going to provide a summary of some of the recent progress in ongoing clinical programs and conclude by outlining additional Foundation initiatives underway to accelerate this progress.

Clinical development for retinal degenerative disease treatments has accelerated impressively in 2019. Both current and past funding from the Foundation continue to play a leading role in advancing the field, especially in moving new therapies into and through clinical trials. Approximately three dozen treatments for retinal diseases, including select dry age-related macular degeneration - AMD - treatments are currently in human studies.

I'd like to highlight some of that recent clinical progress:

Firstly ReNeuron, which develops cell therapies using human retinal progenitor cells. These are stem cells that have almost matured into photoreceptors, the cells in the eye that respond to light and make vision possible. The therapy consists of a one-time subretinal injection of the cells into the eye. As Ben mentioned, ReNeuron recently reported on their ongoing Phase 1/2a clinical trial designed to evaluate the safety, tolerability and preliminary efficacy of their retinal progenitor cells in patients with advanced RP.

To date, 22 patients have been treated in the study, consisting of 12 patients in the Phase 1 segment of the study and 10 patients in the Phase 2a segment of the study. Eight out of the 10 Phase 2a patients treated have reached at least the one month follow up time point. The data show the injection has a good overall safety profile and have demonstrated vision improvement in visual acuity in these patients. The

Company will continue to monitor and report on the longer-term follow up data from the trial. The ReNeuron retinal progenitor cell technology was developed by Dr. Michael Young at Massachusetts Eye and Ear, who received funding for both pre-clinical and key translational studies from the Foundation Fighting Blindness.

(1) Applied Genetic Technologies Corporation (AGTC), is a biotechnology company developing gene augmentation therapies for several different retinal diseases. Their gene technology is designed to use the AAV virus to reintroduce into the diseased retina a normal copy of the gene that has been mutated, to restore visual function. AGTC has reported favorable safety profiles and evidence of efficacy for participants in its gene therapy clinical trials for X-linked retinitis pigmentosa (XLRP) caused by mutations in the RPGR gene, and achromatopsia caused by mutations in the CNGA3 or CNGB3 genes. The Foundation funded critical research for these gene therapy trials and, as Ben mentioned, AGTC used the Foundation's My Retina Tracker registry to identify and recruit patients for their clinical studies.

(2) Next, I'd like to mention PRO-QR. ProQR, is a company that develops a different kind of genetic technology. Instead of injecting a whole new copy of the mutated gene into the retina, PRO-QR seek to correct how the cell expresses the mutated gene. To do this, they inject small DNA molecules into the vitreous of the eye – the fluid in front of the retina. These DNA molecules are very specific to a given gene and a given mutation and change the way the mutated gene is expressed to make it more normal. Recently PRO-QR announced positive top-line results from its Phase 1/2 dose range finding, first-in-human trial of Sepofarsen, which targets the Cys 998 mutation in the CEP290 gene, the gene responsible for Leber's congenital amaurosis 10 (LCA10). In their Phase 1/2 trial, 60 percent of patients had improvements in visual acuity and improved their ability to navigate a mobility course. Based on the positive results in the Phase 1/2 trial, ProQR have initiated their Phase 2/3 clinical trial, called ILLUMINATE, to study the safety and efficacy of 2 different drug doses, over 24 months, in 30 patients.

ProQR also announced that they have started a phase I/II study using their technology to treat mutations in a small part of the USH2A gene called exon 13, where the most common USH2A gene mutations occur. Mutation of the USH2A gene can cause RP or Usher type 2a syndrome. This clinical trial will study 18 people over 24 months of treatment.

As with Sepofarsen, the treatment is intended to slow or potentially reverse vision loss. The Foundation is investing up to $7.5 million through its RD Fund to support the early stage clinical trials of this USH2A therapy.

ProQR has also just announced the initiation of another phase 1/2 clinical trial to treat

autosomal dominant RP (adRP) due to a common specific mutation in the rhodopsin gene called P23H.

(3) Finally, I would like to mention that Allergan, a global pharmaceutical company, which has a strategic alliance with Editas Medicine, a developer of gene-editing therapies, have begun patient recruitment for a Phase 1/2 clinical trial for the Cys 998 mutation in the CEP290 gene that causes LCA10. The therapy consists of a one-time injection into the retina of an AAV virus that carries the CRISPR/Cas9 gene editing technology. Unlike the gene augmentation therapy AGTC are using, where a good version of the mutated gene is being put into the eye, CRISPR/Cas9 gene editing is intended to correct the mutation in the CEP290 gene - you can think of it as a molecule deleting the mutation and then retyping the correct code it – like you do on a computer.

While Editas and PRO-QR are both targeting the same gene and mutation – the Cys998 mutation in the CEP290 gene - their approaches are very different. While the PRO-QR therapy involves regular injections into the eye every 3 months, the Editas therapy uses gene therapy for a one-time, permanent change to the cell's DNA. This highlights how there can be several different ways to treat a given retinal disease, and is a great example of how developers of new technologies are attracted to the retinal diseases to test and prove their technologies.

All of these developments reflect the momentum that is building as we move from basic scientific research to patient participation in clinical trials. One of the key requirements to moving promising clinical programs forward is the ability to find the right patients to enroll in the clinical trials.

To accelerate this, the Foundation created the My Retina Tracker Registry.

This is an online, patient driven, database, that anyone with a retinal disease can join to help accelerate research and clinical trials. The Registry currently contains almost 14,000 participants who have shared information about their disease and is being used by multiple researchers and pharma partners. Common uses are to enroll people with an inherited retinal disease into a focus group - to understand how their disease affects their life and what meaningful benefits they would like to gain from a therapy; as well as for enrolling in natural history studies and clinical trials.

With the increase in gene and mutation specific therapies entering the clinic, understanding the gene causing the disease in each patient has become more and more important. But genetic tests are expensive and often not covered by insurance. Since 2017 we have offered a comprehensive genetic test to members of the registry, at

no cost. To date over 6,500 people have been tested. However, only ~300 clinicians were authorized to order the test, limiting access for patients.

We are very excited about a new initiative that built on that genetic testing study. Last week we were proud to launch a new nationwide program that allows any clinician able to diagnose an inherited retinal disease to order a no cost, comprehensive, genetic test and genetic counseling, for their patients. Importantly, people no longer need to be members of the My Retina Tracker Registry to be eligible for this test. Any person with an inherited retinal degenerative disease may be eligible. This initiative is undertaken in partnership with Blueprint Genetics, a leading genetic testing company and InformedDNA, a leading provider of telephone based genetic counseling to explain simply the complex results of the test.

Our goal over the next five years is to have 40,000 Registry members, with more than 20,000 with genotype information available.

Our Registry is key to facilitating the research that we fund.

Thanks to our successful fiscal year 2019 of fundraising, the Foundation was able to commit an additional $6.5 million for 14 new research projects for inherited retinal diseases, bringing the total number of grants currently funded by the Foundation to 81 projects.

As we continue to evolve the organization and prepare for future success, we have initiated a new 5-year strategic plan that includes $105 million in funding over five years for critical scientific projects. This is in addition to the funding provided by the RD Fund. Here are some of the key components of our five-year scientific plan:

We are continuing our individual investigator award program, clinical career development award programs and multi-investigator collaborative program project awards.

We are establishing a new age-related macular degeneration initiative to advance research in our understanding of the mechanism of disease in the early stages of AMD and identify biomarkers that may be of value in clinical trials. This initiative is made possible by a generous partnership with the Free Family Foundation and will see a total of $3 million committed over 5 years to fund multiple programs and investigators.

There is approximately $28 million targeted for Translational Research Acceleration Program (TRAP) awards, that accelerate particularly promising therapies towards the clinic, with increased funding and mentorship. This is a flexible program that supports academic researchers as well as small biotech companies, where our funding can make

an impact. Awards can vary in length (1-3 years) and funding ($200-500K/year) based on need.

In selecting recipients of awards, we will continue to be guided by the critical reviews and guidance provided by our 50-strong international scientific advisory board composed of the leading researchers in inherited retinal diseases. Our programs are particularly focused on supporting new technologies, identifying new endpoints for clinical trials, and seeking therapies that go beyond treating a disease caused by a mutation in a single gene, to develop single therapies that will treat multiple different genetic diseases – often referred to as pan-disease therapies.

We recognize that there is a critical need for better preclinical models of disease, especially for diseases like Stargardt disease and Usher syndrome, where there are no good animal models to develop and test promising therapies on. To this end we are doubling our funding of resources to develop better models.

We plan to complete the ongoing natural history study of disease due to mutations in the USH2A gene, called the RUSH2A natural history study, by 2022. Our Clinical Consortium also plans to initiate at least 3 additional natural history studies in the next 5 years.

We are currently launching a natural history study of RP caused by a mutation in the EYS gene. The study, called PRO-EYS, will follow approximately 100 patients over 4 years, with annual clinical evaluations. The goals of the international study include estimating the rate of disease progression and evaluating the usefulness of various outcome measures for future clinical trials for emerging therapies.

At least one of the 5 natural history studies will seek to identify useful clinical endpoints and characteristics that are common across multiple retinal diseases, rather than the single disease focus of past studies.

And finally, as I already highlighted, we will be expanding My Retina Tracker Registry and the genetic testing program.

In summary, our five-year plan encompasses the full spectrum of funding programs from early translational research to clinical studies, the fundraising and revenue required to fund the highest levels possible, and the communications and education plans that allow us to be as connected as possible with our constituents.

I’d like to turn the call back over to Jason.

Jason Menzo, Chief Operating Officer:

Thank you very much Brian. That was a great review. What we'll do now is open up the lines to take your questions help remind us how to do that is Chris, can you review the 3 different methods for folks to ask questions.

Chris Adams, VP, Marketing & Communications:

As a reminder there are three methods for asking questions. First, you may access the Q and A feature on the bottom of the Zoom control bar and type in your questions. Secondarily, you can ask questions verbally. To do so, please click on 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 and are not in the Zoom app, please submit your questions via email at info@fightingblindness.org.

Jason Menzo, Chief Operating Officer:

While we are compiling questions, I'd like to take a moment to remind everyone about some of the resources that the Foundation provides. Our website, FightingBlindness.org, our Facebook page, Twitter, LinkedIn and Instagram accounts are all great resources for learning about the latest developments in the retinal degenerative disease space. If you have specific questions about your diagnosis, disease or genetic information, you can always find more information on our website under the Newly Diagnosed or Retinal Diseases sections. We can also help you connect you through our physician referral program, which is available on our website under the Resources section. You can also reach out to us by just sending an email to info@fightingblindness.org.

We're in real time, looking at the chat feature from the Zoom application and also, receiving and sharing any questions that come in via email. While we're compiling our questions, I would like to take a moment to remind everyone about the resources we have including our web site - we launched a brand new version at fightingblindness.org. Our Facebook page, our Twitter and LinkedIn accounts - these are great resources for learning about the latest developments that are happening in the world of inherited retinal diseases. Also, I want to remind folks that if you have a specific question about your particular diagnosis, or genetic information, or if you would like to learn more about referrals to physicians - these are all resources that we have here at the Foundation.

For example, we have on our web site, a newly diagnosed section where there's a ton of information on each of the different conditions and in the retinal disease space. We

have the retinal disease referral program, under the Resources section, and always you can contact us at our general mailbox, info@fightingblindnesss.org. We'll focus on taking questions that are more general and affect multiple people, so when we do get questions that are specific, to an individual diagnosis or individual's genetic information, we tend not to answer those here on this call, because there's so many folks on the call. We want to be able to provide you with those answers and resources, so we'll do that offline. With that, we have a couple of questions that have come in.

The first handful of questions are all specific to retinitis pigmentosa. I'll direct these to Brian. Maybe you could do a quick review of the currently approved treatments, such as Luxturna, and also a high level review of treatments that are in development specific to RP.

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

Thank you Jason, Yes, I would be happy to. So as Jason said, Luxturna is for gene augmentation therapy for LCA2, which is due to the RP65 gene. Approved in late 2018, that is now the main approved therapy which is available on the market.

There are no other specific therapies approved for RP. There are a number of prosthetic devices such as the August system, IRIS 2 system, the Alpha IMS system, these however are devices which are used for people who have lost most of their vision.

They restore a level of functional vision but they do not have the same impact as gene augmentation. There are a lot of clinical trials going in the RP space.

Many of these are gene specific. So we have a number of gene specific therapies for RP such as for the PD6 beta gene, which causes the retinitis pigmentosa. There are other therapies which are more PAN disease, which may address RP. One that is in clinical trial that Ben mentioned was Nacuity product, NACA, that is an antioxidant to slow or stop progression of the disease.

And then as we, talked about earlier today, there are some therapies such as Pro-QR's technologies, for treating autosomal dominant RP.

And further back in the research pipeline, we have a number of gene editing approaches to address specific genetic diseases that cause RP.

And, so there's a lot of activity in the pipeline, if we look even further back, the Foundation is continuing to fund a lot of PAN disease therapies. For instance, this year we were very excited about an application of a small molecule that was able to increase

the survival of cells in RP, that we have funded with one of our translational research acceleration programs. So there's a very large number of programs, in progress to support RP.

But, there is only the one approved program at the moment, which is Luxturna.

Jason Menzo, Chief Operating Officer:

Okay. Excellent thank you Brian. There are two other RP related questions that make sense to address right now.

The first of which is coming from a couple of folks here today, but also has been a question that has shown up on email to the general mailbox the last couple of weeks, which is about cataract surgery and whether or not there are special considerations or any guidance for individuals that may have additional complications from cataracts.

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

I need to be cautious here because I'm not a clinician and I cannot give clinical guidance, as I'm a researcher.

My guidance here would be to talk with your clinician, about that particular concern.

My understanding, as a scientist not as a clinician is that, cataracts do not normally create a problem for your eligibility to these therapies being developed. Cataract surgery is a very common procedure now that I think can be done either in association or, early or later with the therapy, if you may be a candidate. Talk to your clinician about concerns like that - they're in the best place to give you the best advice.

Jason Menzo, Chief Operating Officer:

The last question in this section I'll address to you Brian and then we'll move to the next grouping of questions. There have been have a couple news articles and a couple questions, about alternative potential treatments - sometimes you hear horror stories in the general media - coming through less proven channels, than the FDA regulatory path with regards to the any retinal disease. Without getting into any specific programs or web sites, maybe you could just speak to generally things that fall outside of the area, review and expertise in terms of potential treatments folks may promote.

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

Yes, that's a very good point I think that some of the common therapies in this field may revolve around, for instance, cell therapies or STEM cell therapies as they're called.

And, it is really important for anyone when they're thinking about being involved in a clinical study or a clinical trial to talk to their clinician and find out, if this is a good study to be part of.

Now, generally good studies, are going to have their study protocol registered on a government site called clinicaltrials.gov. And so if you go on there you can look up the details about the study - who is conducting it and who is sponsoring the study.

But you have to be aware that there are trials out there which are not being done in a well-regulated manner. The thing that you really need to be aware of, is whether the study that you're thinking about, has been approved by the FDA as a clinical study related to your disease.

Now there are many treatments and therapies out there that have good stories about good outcomes. They sound as if they should work - they sound logical to a lay person. But they are not approved by the FDA and they do not have what is called an IND (Investigational New Drug).

So again this is something that your doctor will be able to give you clear advice on. You must ask if there's an IND, if it is a genuine clinical trial, which is approved with oversight by the FDA, where they have already determined the safety of the treatment, and they believe that it is a reasonable thing for you to try.

Jason Menzo, Chief Operating Officer:

Great. Thank you very much Brian.

Let's shift to a couple of questions here that I want to direct to Ben. On our last Insights Forum call, we talked about the natural history study that we're going to begin enrolling the EYS gene. Can you give an update on that program?

Dr. Ben Yerxa, Chief Executive Officer:

So making a lot of progress on the EYS natural history study - in fact we plan to initiate and announce the start of that trial, before Thanksgiving. So, should be in a few weeks we'll be up and open and getting started. We're excited about that.

>> SPEAKER: Okay. Um, couple of questions here about the RD fund, um, there's a -- pretty comprehensive overview of our current portfolio with the RD fund.

Um, but a couple of folks were asking, what are we looking at next and what are some of the things in the pipeline we're evaluating for the RD fund maybe you could speak to that?

Dr. Ben Yerxa, Chief Executive Officer:

Great question. You know, when the RD fund now we're, really calling it fund one as little over $70 million, we're about two thirds committed with our capital.

So, realistically we can make between 2 and 4 more investments in that current fund depending upon the size of the investment. So because of that we're actually strategically thinking about, creating a fund two, hasn't been you know, fully approved yet but something we're talking about, internally, that of course, that will require major capital campaign to get that funded. But that's something we're thinking about, because, we do feel like in is a very power full financial vehicle to both move the field forward to get clinical results, and also, have a potential long term financial stability for the foundation.

Um, other than that, in terms of what else is new you know I think, Brian mentioned the new translational research acceleration plan.

That program, which we've done in the past, but we made some enhancements to that, so that it can now be more integrated with the fund activity I think that will, help with the acceleration and through of the clinic into the exploring.

Jason Menzo, Chief Operating Officer:

We have somewhere in the neighborhood of 30 questions that have been chatted in or typed in the email. We talked in the financial review, that we raised about $56 million in fiscal year 2019. But the budget for this fiscal year we're in right now, calls for revenue in the neighborhood of $25.6 million. So the question is, why such a drop off year over year in terms of revenue performance? There's a real simple explanation that we actually had two unique gifts that came in this past year that were specifically earmarked for the RD Fund that were in the neighborhood of $24-25 million from the Gund family challenge from several years ago. So there was a schedule associated with some big dollars that came in this past year that came in on schedule.

Looking at the revenue predictions for this year, those unique events are not anticipated to repeat, and so, what we do in budgeting each year is that we take a look at previous pledges and other unique large one time initiatives that have the potential to bring in big revenue, and then, normalize that on a forward looking basis for the year that we're in.

So we actually have a five year budget with revenue projections all the way up to 2024 and obviously we would love to be wrong on that, where we raise much more revenue than we're anticipating. That's how the delta from this last year and this year's revenue is explained. The other question that I got, was about the public service announcement, the PSA campaign. And there were a couple of folks that asked where they could view, listen to or be exposed to that campaign.

We do have it up on our Facebook page and on our YouTube channel. It is up on our web site as well.

We have it up in the handful of places. Actually Chris, maybe you could speak to if there's an opportunity that we have planned to push it. I think we did send it out to the entire community. We're proud of the work - it featured a handful of folks from within our community who are very special advocates for our cause. And, it was really great to feature them in the video.

Chris Adams, VP, Marketing & Communications:

Just as a reminder it is available on our YouTube channel. If you do go to our web site, right at the top of the page, where it says Join the Fight there is a link that will take you to directly to the page that features our video and, it has a short bio of the individuals from our community who participated.

Jason Menzo, Chief Operating Officer:

Okay let's shift to a couple of questions on Stargardt. Let's get the 30,000 foot view of the current progress from a clinical standpoint.

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

Yes, certainly, the disease is a very interesting disease, because we know quite a bit about it are and there are many different points of intervention - so many different types of therapies.

At the moment we have a Phase 3 study under way with emixustat, an oral drug. Developed by Acucela, the drug works by slowing the buildup of toxic waste products that lead to retinal degeneration in a number of retinal conditions, including Stargardt disease. As Ben mentioned, it's showing efficacy in the early read out in the clinic, There's a drug in a Phase 2 clinical trial, which is a modified version of vitamin A which is part of the light sensing structure in the eye. Research going on in a number of different groups. The Foundation is currently funding innovative work using nano particles to deliver the treatment to the retina, and also some, gene editing work in the

Stargardt disease. So there are a lot of clinical trials going on, and our research portfolio is full of varying therapeutics.

Jason Menzo, Chief Operating Officer:

Thank you very much Brian.

Let's go to the phone line. Hello - please feel free to ask your questions.

Participant:

I had a question with regard to Usher syndrome specific to 2A gene and was wondering if I could get more information on what clinically is being done with that particular gene and, any gene therapy that might be going on with that research.

Jason Menzo, Chief Operating Officer:

I'm glad you asked that question as a handful of folks have asked similar questions.

Dr. Ben Yerxa, Chief Executive Officer:

The best thing to do is send an email to the Foundation at info@fightingblindness.org so we can connect you with the right information and you can check it out.

Participant:

Okay thank you very much for that information I will do that.

Jason Menzo, Chief Operating Officer:

There was a question that is a really important question that has been asked, not only on this call here today, a couple of times, but also, I would say over the last several years. This is related to mental health and does the Foundation have a specific support for patients or family members. I am really happy to say that, recently, within the last 8-9 months we have formed our Strategic Council. It's a group of younger professionals, in their late 20s to early 40s who are in the inherited retinal disease community. Mental health is a topic that came up in our Strategic Council.

There's a small group of folks from within the Council who are specifically tackling this question and working to think through, how we as a Foundation can provide the appropriate support from a mental health perspective. We do think it's a very important topic and something we're actively working through to identify the right resources that we can provide to our community, so thank you for that question.

Jason Menzo, Chief Operating Officer:

We have a question from our friend in Bentonville, Arkansas. She is asking for an update on the clinical study of gene therapy for RP.

Ben, you were really close to that - can you speak to the update.

Dr. Ben Yerxa, Chief Executive Officer:

SparingVision is developing SPVN06 breakthrough technology gene independent for the treatment of RP. We won't know more specific timing until -- they get, to their regulatory filing. Right now it's late 2020 for the trial be conducted in Paris, France and one other U.S. site. So you have to stay tune for more details as we get into next year.

Jason Menzo, Chief Operating Officer:

Excellent. We have around 20 plus questions that have been typed in, that we have not had an opportunity to get to yet.

As we have done with previous calls, on our prior Insights Forum, we take all the questions that we were unable to get to, and we will review them here at the Foundation internally and we'll get back with you individually.

We would like to thank you all for participating in today's call. We really appreciate everyone's engagement throughout the call and look forward to our next Insights Forum call. Thank you and have a great day.

A submitted question, answered after the call:

Participant:

Do you anticipate female enrollment in the trials for x linked RP with the RPGR gene?

Ben Shaberman, Senior Director, Scientific Outreach & Community Engagement:

Good question. All the trials are currently for males only. Perhaps the investigators will include some females in Phase 3 trials, if the emerging therapies reach that stage. Stay tuned www.FightingBlindness.org for updates on these trials