Foundation Fighting Blindness
***Insights Forum* Call Transcript**
July 24, 2019

Jason Menzo, Chief Operating Officer:

Good Morning. My name is Jason Menzo and I am the chief operating officer at Fighting Blindness. I would like to thank you for joining the call. 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 which is to drive and accelerate the search for prevention, treatments, and cures for retinal diseases.

On today's call, our CEO, Dr. Ben Yerxa, will provide a strategic update and I will follow with an operational and financial review. We are also very pleased to have a special guest speaker with us today, Congressman Sessions, who will highlight the exciting work advancing in Congress related to the creation of Eye Bonds which are financial instruments to raise money for research specifically related to a broad range of blinding eye diseases and conditions.

Congressman Sessions served 21 years in the U.S. House of Representatives and is a passionate advocate for initiatives related to saving and restoring vision to the 4 million adults and almost half-a-million children in the United States who are blind or with severe vision impairment.

As we have in the past, we will have a question-and-answer period at the end of the call, and the operator will provide instructions on how to ask your question at that time. A replay and fully accessible transcript of this call will also be available on our website in the weeks ahead. We are committed to making this call and all of our activities fully accessible to all participants, and to that end, this call is also being closed captioned. If you have any feedback related to accessibility or any other suggestions for this call, please reach out to us directly at info@fightingblindness.org.

I would like to turn the call over to our CEO, Dr. Ben Yerxa.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you, Jason. Good morning, everyone, 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 and macular degeneration. We are the catalyst in funding breakthrough research and innovative science that provides preventions, treatments, and cures as fast as possible.

I would like to highlight some of the developments both at the Foundation and within the broader community.

First, we continue to make excellent progress growing the My Retina Tracker patient registry and the Genetic Testing Study. There are currently over 12,400 registry members with a profile, and a total of 23,300 members. Of these, over 5,200 have submitted a test requisition for the genetic testing study and over 3,900 genetic test reports have been returned. This is fantastic growth for the registry.

On the research front, we recently completed enrollment in the Usher syndrome type 2A Natural History Study, called RUSH2A, and are planning our next related study, called PRO-EYS, which will follow approximately 100 patients with the EYS gene for four years, with annual follow up visits. This study will be chaired by the highly regarded and experienced, Dr. Mark Pennesi, Assistant Professor in Ophthalmic Genetics at Oregon Health and Science University, Casey Eye Institute. We're in the process of completing contracts with sites and reading centers and expect to begin patient recruitment for the study enrollment in the fall of 2019.

We've also been exploring potential natural history studies in other IRDs and opportunities to partner with industry on studies through our Clinical Consortium. Stay tuned for that.

One of our recent major initiatives is expanding translational and clinical research projects for treatments and cures through our retinal degeneration fund, or the RD Fund. Launched late last year, this retinal disease venture philanthropy fund drives emerging therapies that are moving toward, or in, clinical trials. The RD fund is part of our strategy for adapting to a rapidly changing environment where

we are seeing many more projects ready for translation and simultaneously the cost of the R&D work is increasing.

The fund, which now has more than $70 million in initial funding, invests in companies with projects that can be in clinical testing in less than 18 to 24 months.

The RD fund continues to review many exciting investment opportunities for companies and programs, and we look forward to sharing the new funding deals as they are finalized. To date, five investments have been made totaling approximately $28 million in currently committed capital. Our most recent investment hasn't been publicly disclosed yet, so you will have to stay tuned until that is announced. We will provide updates on the progress of our portfolio companies during the next quarterly call.

To conclude my update today, I would like to highlight a recent article describing the Foundation's contributions to genetic research, which was published in the peer-reviewed journal, Genes. The article provides a comprehensive overview of the substantial progress made possible by investigators and the patients and families who generously support their groundbreaking efforts. The paper has been downloaded nearly 500 times in two weeks. The Foundation's long standing, guiding imperative is to ensure that research is based on good science, resulting in more preventions, treatments, and cures for IRDs across the finish line for everyone affected. You can find a link on the Foundation website under the section called "Eye on The Cure Research News".

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

Jason Menzo, Chief Operating Officer:

Thanks, Ben. What I'm going to do is provide an update on some of our key operational initiatives.

As many of you know, our all new FightingBlindness.org website has been live for a few months, and the impact on engaging our community is notable. Our team is working hard to provide compelling new content, including research updates and what we call Beacons of Hope stories that highlight amazing stories of individuals

in our community doing great things with their lives. These stories are published bi-weekly and we expect to continue to add new content, new features, and accessibility improvements to our site in an effort to make it as engaging as possible.

Our social media channels also continue to grow as we expand our content with research updates, Beacons of Hope stories, and also Healthy Vision Month, which happened in May, awareness and fundraising through VisionWalks, AmazonSmile and eBay for charity. Speaking of Amazon, did you know you can use Alexa to make a donation to Fighting Blindness? Ask her to make a donation to Fighting Blindness and check it out.

We have spent several months preparing for and just recently launched our brand-new online fundraising platform called Classy. The team has spent nearly six months working directly with the product team from Classy to ensure best-in-class accessibility for our community. While we're not at the finish line with those accessibility features just yet, we're pleased with the features that we have here at launch and expect the platform to get even better in the months ahead and are looking forward to getting feedback from you, who are using the platform.

Additionally, we continue to enhance our staff. This month, we added Judy Taylor as Vice President of Development for the team. In her new role, Judy will be responsible for the strategic direction, planning and oversight of our Major Gifts and Planned Giving functions for the Foundation. Judy is based in Columbia, Maryland and brings over 25 years of experience developing innovative fundraising programs, and engaging donor audiences. She knows the Foundation very well, and our stakeholders as well, because she served as one of our regional development directors for six years, beginning in 2006. We're excited to have Judy in the expanded role, enhancing the talent on our team and the group of development professionals we have.

Finally, I'd like to conclude my remarks today with a brief summary of our financial position. As a reminder, the Foundation operates on a fiscal year that runs from July to June, so we completed fiscal year 2019 and our audited fiscal year-end financial statements will be available later as we get into the fall. That said, I'm happy to share with you today some of our latest projections for the year. But please keep in mind these are just projections and the final audited

numbers may vary slightly. Our current projections for the year are total revenue of $25.6 million dollars against an operating expense of $13.4 million dollars, which yields a surplus of $12.2 million dollars. This projected surplus is favorable to our original budget and gives us more resources to fund the great science in our brand new strategic five-year plan. We are pleased with these financial results and believe our 2019 performance will reflect favorably with various non-profit performance tracking websites and we're starting to see positive rating updates with some of those sites including Charity Navigator.

We are very grateful for the generous support of all of our donors who continue to support us here at the Foundation each year through our events, annual giving and gift efforts. We are deploying these funds as efficiently as possible into cutting edge research.

It is my distinct honor to introduce, Congressman Pete Sessions. He has been a strong supporter of the Foundation and our work, especially as it relates to the Eye Bonds legislation that has been introduced in the House of Representatives. H.R. 2620, which is also known as the Faster Treatments and Cures for Eye Diseases Act, is bipartisan legislation to fund treatments and cures for blindness and other forms of severe vision impairment. The bill will establish a five-year pilot program for the establishment of Eye Bonds to raise money for research for a broad range of eye diseases and conditions. This legislation was originally introduced in July 2018 and with the convening of the new Congress in 2019, the bill was recently re-introduced as part of the standard legislative process. We've asked Congressman Sessions to share some highlights with you all today, so I will now turn the call over to him. Congressman Sessions.

Congressman Pete Sessions:

Thank you very much and what a pleasure it is for me to join each of you in this very important update for Foundation Fighting Blindness.

First thing I want to say is that the legislation which we're going to speak about today is, as was noted, H.R. 2620, is a bipartisan piece of legislation whose cosponsor happens to be the member in the majority party who handles appropriations for the National Eye Institute and the National Institute of Health,

NIH. We have a very good sponsor in Sanford Bishop, who is a very dear friend of mine, I've known for many years, and he is committed to this piece of legislation and the surrounding ideas that will enable us to have more money, more power to make more investments and to get better answers.

Second, I want to say for those of you who did not have an opportunity to come to the latest Washington annual event, it was a very exciting event full of not only hope and promise but opportunity and enlightenment of people who are struggling with and succeeding quite well with eye diseases. I serve as a parent. I've been involved for the last 10 to 12 years in this effort, and was the primary contact for the Foundation on the Hill, not just for photo receptor cell development, but also in working with Francis Collins, the former head of the NEI. So, I have a rich history in this and believe that we have people on the Hill who see this as a very, very important process.

The purpose of the Eye Bonds is to spur private-sector investment in translational research and to give advanced treatments and cures for blindness and other causes, a better chance of not only seeing the light of day but becoming reality for each of us.

So, when approved by Congress, the plan is that the new bonds would finance packages of loans that would total up to one billion dollars of new projects over five years of the pilot program. This is an opportunity for us to further enable and help those people that might not be fully funded by NIH or through NEI to receive the opportunity to put their best ideas forth. Underwriters to the bonds would determine how many projects they would fund and also what amount, not to exceed $250 million in a year. The National Eye Institute as part of NIH would evaluate these programs eligible for funding so it would be in essence a public/ private partnership. Eye Bonds would not be replacing the existing federal funding but would be a supplement and an addition to those efforts.

Eye Bonds would be purchased, we believe, by mostly pension funds, insurance companies, and other institutional investors who see this as not only a good thing because they are involved in what is a large social and medical issue, but more importantly it would lend to making America and the world a better place by solving some of the key issues related to blindness. Money generated would then be put into scientific projects which were recommended by the National Eye

Institute, and when the bond matures or comes due, the scientific project would have reached a stage of development where it has commercial value. In other words, we're trying to spur innovation, public/ private partnership to get more researchers, more investigators who would come not only to institutions that would find the cure but really to bring them in from basic science and to add a whole lot more people who were ready to take on this task.

The legislation, as it is, holds enormous promise, I think, and because it offers a real opportunity for not just people like me, family members, but people who are involved in their own impairment to see that we are going to take on and accept this challenge and win. As you are each aware, we have a large number of people, veterans of the Iraq and Afghanistan wars, who have been hurt as a result of the war and suffer sight issues. This is another important issue of social consideration for us to do these Eye Bonds.

Eye Bonds are going to fill a critical need and we believe there are many promising therapies that today, simply do not get out of the lab, do not get into a clinical trial, simply because of money, of funding. We think that there's a case that rare diseases or conditions where we can provide proven regulatory pathway, they could become successful. Eye Bonds would be vital to this research, we believe, at minimal risk to the taxpayer and a huge advantage to the government. I believe in working with the Foundation Fighting Blindness over these years, that we have to bring new innovative techniques and ideas to the marketplace to get more money. We have done a number of things where we have worked with NIH to make sure we are included in other translational and other ideas, as you heard, because they have pots of money and we find that these problems that also include the mind and the brain, all of these come to bear with this Eye Bonds issue. We have currently some 15 members of Congress who are cosponsors. We do not have a Senate bill at this point but anyone that would like to be aware of that can find a way, I'm sure, Alexa can go to the HR bill which we've given you, HR 2620, which takes you to the House of Representatives website. HR for house resolution and then you can see the 15 members who have signed on. I will tell you that we would ask each of you join in by contacting your member of Congress, your House of Representatives member of Congress and ask that they not just cosponsor this, but they understand the important issue that is related to these Eye Bonds and the long term success not just for

Foundation Fighting Blindness in meeting its mission, but more importantly in conquering the issue of retinal diseases. Thank you very much, I'm open for any questions you may have.

Jason Menzo, Chief Operating Officer:

Thank you very much, Congressman Sessions. We will open the lines for Q and A in just a few minutes. I cannot thank you enough on behalf of the entire Foundation Fighting Blindness family, Congressman Sessions, for your support - not only on your hard work over the years for this bill, but also all of your support as it relates to issues related to inherited retinal disease and specifically the Foundation Fighting Blindness. We're grateful to have you as part of the family and look forward to working with you in the years ahead. We will open the lines; can you provide the instructions, Chris.

Chris Adams, Vice President, 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:

Thank you, Chris. While we are compiling questions, I would like to take a few minutes to remind everyone of some of the great resources here at the Foundation. First of all, we mentioned earlier in the call, the all new www.fightingblindness.org website. There's a ton of new resources on the website. We have our Facebook page, our Twitter page, LinkedIn and Instagram accounts and those will help you find out about the latest developments in the retinal disease space.

If you have questions about your particular diagnosis or genetic information about you personally, you can find out more information on our website. We've created a brand-new Newly Diagnosed section and there's the Retinal Diseases section where there's a lot of information. We can also help connect you through our physician referral program which is also available on our website under the Resources section. And of course, you can always reach out to us directly by sending an email to info@fightingblindness.org.

To find out other information about your diseases or genes, go to our website and look under the News and Research section in the drop-down menu where you will find a lot of information, blogs, newsletters and other informational materials about current research, that is updated very frequently.

Finally, one of the new enhancements on our website is similar to the resource available at clinicaltrials.gov and very shortly we're going to be launching this new resource which will have a pipeline that includes all of the latest research advancements and information on clinical trials happening in any area of the inherited retinal disease space. It will be a really nice resource for folks in addition to what is available at clinicaltrials.gov.

We have some questions now. Why don't we first open up to the phone lines. If there is anyone that has a question on the line, I see a couple of folks have their hands raised. Chris, is there anyone you would like to unmute their line to ask a live question?

Chris Adams: Yes. Linda, you have a question.

Linda (Caller): Yes. Can you hear me?

Chris Adams: Yes, we can.

Linda (Caller): Good. So, my diagnosis is Optic Nerve Syndrome. I have contacted you guys in the past, probably a few years ago, and I am frustrated because I can't seem to get information. Of course, the Eye Bonds would be great if we could get some research on this, because every time I go to the doctors, they always tell me they just don't do any research on my issue. And so of course I'm going blind

and literally I have no resources, so I don't know what to do. I wanted to know if there was anything that you could share with me on that disease since I don't have retinal issues.

Jason Menzo: Hi Linda, this is Jason. What I recommend we can do is have an offline conversation. I think depending on your area we could probably certainly get you information specific to your condition but also what would be a great resource is we can connect you with the local retinal specialist expert that can certainly take the ball and run with it. If you don't mind, you can shoot an email to info@fightingblindness.org and we will contact you directly off line to talk through that.

Linda (Caller): I don't have retinal, it's not my retina. Why would you want to send me to a specialist?

Jason Menzo: Once we get more background, we can certainly do whatever we can to help assess that.

Linda (Caller): Okay. Absolutely I'll send a message to the email.

Jason Menzo: Thank you so much.

Jason Menzo: There's a question about VISIONS2020. We see it was just chatted in. Thank you very much. The answer is yes, there is a VISIONS Conference in 2020 that is planned. It is planned for Minneapolis Minnesota in June of 2020.

Dr. Ben Yerxa: June 18 through the 20th.

Jason Menzo: We will start promoting this on all of our social media and websites later this fall to get the excitement up for VISIONS2020. The VISIONS2020 registration will probably open around January. But those of you on the call have the inside track, June 18th through 20th in Minneapolis.

We have several questions, Congressman Sessions, about what the road map looks like for Eye Bonds now that it has been introduced to Congress and we have the 15 co-sponsors. You gave great guidance today on what everyone on this call can do to help try to get more co-sponsors and raise awareness on how important

this issue is. Can you give commentary about what the road map from where we are today to down the path may look like as it relates to this being put in motion?

Congressman Sessions: Yes, I can and thank you very much. It is a longer path, it's not a shorter path. There's no shortcut. It's going to require a number of things and one of them is it has jurisdiction in some committees that it has to become important to them. Congresswoman Kathy McMorris Rogers sits on Energy and Commerce. They have part of the jurisdiction as well as services. It is split jurisdiction that we have that we are working with. What does this mean? This means it's going to require us to go and sell this to members of Congress. There literally needs to be a threshold of performance whereby a number of members need to be aware of it where you can then convince a committee chairman or subcommittee chairman to have a hearing. The hearings are expected to vet out ideas, they are expected to understand what would be gained from this. So, we're in the initial phases. When I was a member of Congress and began this, my major task was to make sure that I got the jurisdictional elements that would be taking care of. There were some objections based on financial services because there is an obligation under these bonds that the Treasury Department would be issuing them which means that Treasury Department needs to understand what and why it would be, and what the expected outcome would be. So, we have begun and did the initial spade work a few years ago as these ideas began to take off. You have heard a number of terms, one of them is crowd sourcing, you have heard open sourcing. These are the kinds of terms and terminology that are new to not just the investment community but also to discussions within members of Congress about how we're going to get this done. So, I would say to you that very quickly, if not this year, next year, we will begin a robust effort to make sure that the members focus on these activities. Seemingly, it has become more of a learning process for the new majority rather than a doing process. And I believe Chairman Bishop sees that his timeframe would be the majority of these to pass this bill and get the

legislation in shape for passage next year. Like to see it this year but we've got a lot of work to do.

Jason Menzo: Thank you, Congressman. We really appreciate that update and obviously are optimistic for how this is going to progress. We have a couple of questions related to a specific clinical trial, the AGTC trial. I'm going to introduce Ben Shaberman, who is our director of scientific outreach and community engagement, to give an update since there are several questions about it.

Ben Shaberman: Good morning. AGTC has launched a therapy clinical trial for people with X linked RP. Specifically, those with mutations in a gene called RPGR. Mutations in that gene are the most common cause of X linked RP. The trial has been underway for a while now. They are enrolling 30 patients. It's a Phase 1/2. The primary goal of the trial is to evaluate safety. And they are also evaluating different dosage levels in different patient groups. Most recently, in April, they announced they have expanded to their last group which is a pediatric group. Those patients can be as young as six years old. They have not reported any results, but the trial seems to be moving forward pretty well. And it will be taking place at ten different sites across the U.S. There are many different locations for it. That's basically the news that's been made public in recent months.

Jason Menzo: That's excellent, thank you very much, Ben. There are several questions about a general status of treatment options and the various potential scenarios for treatments, whether it be stem cell potential treatments, gene therapy or other what we call pan-retinal. I'm going to ask Dr. Yerxa to talk generally about the progress on clinical trials and a multiple treatment modality as it relates to inherited retinal disease.

Dr. Ben Yerxa: Thanks Jason. I think it's fair to say there's really a lot of progress going on in the clinical area, regardless of modality. We're tracking about 36 different clinical programs. That number tends to grow over time and we're seeing a very robust pipeline of potential projects enter the clinic in the coming months. We are

seeing a lot of momentum in not just gene therapy but also things like anti-oligonucleotides. We are seeing different kinds of cell therapies, whether it is cell replacement or atrophic factor component that goes with the cell therapy. We're also seeing small molecules. So, essentially, we're seeing growth in the clinical trial area with many modes of attack, which is good. It's a goal and we're tracking these closely. It's a good problem for us to have. It's getting more challenging to stay on top of all of these programs as they enter the. We, like you, anxiously await reports on data and there will be a number coming up in the next six to 12 months.

Jason Menzo: Thank you, Ben. I think it's well stated, that not only are we following clinical trials but, in many cases, we're actively engaged through the RD Fund or through other conversations with many of the biotech companies leading the way in all of these different areas. And certainly, the regulatory path is changing rapidly. We are involved in many conversations across the board to partner and collaborate in driving our mission towards the finish line.

Chris, I see several hands raised on the phone. Why don't we go and answer a few more questions from the phone?

Chris Adams: Sure. Jan Kin has a question.

Jan (Caller): Good morning, can you hear me?

Chris Adams: Yes, Hi, Jan.

Jan (Caller): First of all, I want to thank the speakers for gathering the information. My question is about my daughter. She has recessive Stargardts disease. Being that it's recessive I don't see any trials, I don't know how to go forward and help her. As I understand you have to have genetic testing and we're not too far from Indianapolis, Indiana. I was wondering if there's any free genetic testing, so we know what to do and how to go forward with that.

Jason Menzo: Thank you very much, Jan. Really appreciate the question. As it relates to specifically about clinical trials for the recessive type of Stargardt, we can take that. Generally speaking, as it relates to genetic testing, I'm excited to be able to share with you, and really everyone on the phone, our My Retina Tracker

has free genetic testing associated with it and we can work with you to identify a local researcher who we could partner with. It's an offline conversation we can have to walk you through that process and we're happy to do so. The only thing I would ask to flag it for us would be to send an email to info@fightingblindness.org and we will contact you directly. Thank you very much for the question.

Jan (Caller): Thank you very much.

Jason Menzo: I see we have another three questions on the phone. Teena you have a question this morning.

Teena (Caller): Hello can you hear me?

Yes.

Teena (Caller): This is Teena. Hi, Jason and hello, everyone. I have a burning question regarding what Jason mentioned about the regulatory landscape the eyes POC model which is a global leader in the U.S. is and collaborating at the breech with academic and biotech regulatory as well. Used to be a very good lobby. My question is, has the FDA implemented any conditional system and they have regenerative medicine means they were able to concede early pool and even before just right after POC and there were a lot of talks in the U.S. over the last year, but I haven't got any update as the FDA had any things able to approve the product.

Dr. Ben Yerxa: Hi, it's Ben Yerxa, and I can try to answer if I understood your question.

Teena (Caller): Guidelines from FDA regarding the early approval or conditional approval for rare gene therapy stem cell especially.

Dr. Ben Yerxa: Right, this is Ben Yerxa. I think it's a good question you raised that for rare orphan diseases sometimes the FDA can be more flexible as it reviews data packages. To my knowledge to date, in the inherited retinal disease space, no sponsor has tried a conditional approval angle. But I think that for some of the subsets

of IRDs, very small patient populations and strategies like that would probably make sense given that there's data to support it. So, we're here as a Foundation to help sponsors get these things over the finish line and we're in the unique position at the Foundation where we're not a sponsor, not industry, but we can really work with industry and the agency together to try to get things to patient as fast as possible. We're certainly here to help anyone who needs that kind of assistance.

Teena (Caller): Thank you.

Jason Menzo: Thank you, Teena, and thank you, Ben. We have another question related to Eye Bonds. Congressman Sessions, I will direct this to you. The question is related to the partial government guarantee element of the Eye Bonds legislation and the question is, will that be enough, obviously it's a little bit of your speculation on this, but will that be enough to attract the type of capital to inherited retinal disease or really blinding disease as it's designed. Just share your perspective on how the dollars will be affected to the field based on the current arrangement and setup of the bill?

Congressman Sessions: This is, whoever asked this question is, really pretty smart let's give them credit for that. There was a vigorous debate and discussion that took place internally for a great period of time. And the question was how do we get outside the model to where it becomes competitive with the NIH. We want to push them. We also needed help and the back up from the government and the institutions that are out there that don't really know enough about the risks or the advantages that could be associated with what I would say just a private sector answer. So, we found ourselves very quickly moving to a model that would use the NEI and National Institute of Health, NIH. Let's encourage them because they have projects that today they would want to fund but they have not funded to include those on the list. In that process, the investor would feel like these were good ideas but there just wasn't enough money in it. We believe that the investment opportunity would come about not only from social responsibility because there's a lot

of social clarity that is enhanced across the marketplace today through social activism and this gives those investors a chance to do something great with the backup of the government because they are making sure that these are official projects. So, it became a great model, we hope, of a public/private partnership. This person is right. I wish they had been in the room to add. If they would like to engage in the debate with me, they can email you and you can link them up with me and I'll be very pleased to hear their ideas.

Jason Menzo: Thank you very much. That's excellent feedback. Chris, can you remind people how to ask a question again? We have a couple more in the queue and just to make sure everyone has had an opportunity to ask a question if there is one on their mind.

Chris Adams: Sure. As a reminder, there's three ways to ask questions. The first one is to use the Q and A feature within the Zoom control bar. Also, if you are using the interface you may raise your hand and we will unmute you to allow you to ask your question. And third, if you're only calling via phone feel free to send an email to info@fightingblindness.org. I see we do have a question by Vinod. You are unmuted.

Vinod (Caller): Thank you for taking my call and I really thank you for this important information. I have a question regarding my sister. She is in India and she is 53 years old. And she has very low eyesight remaining now and deteriorated in time. I would like to know if there's any progress on getting the treatment for this and what can we do to make sure the eye condition does not go any further.

Dr. Ben Yerxa: Hi, this is Ben Yerxa. I think a couple of things. One is it's always helpful to know what the genetic diagnosis is that goes along with the clinical manifestation. As trials progress with gene specific therapies that could be important to know. The Foundation is funding some efforts in RP that are essentially gene agnostic. They just haven't quite made it into broad clinical trials. If you want to talk offline, we could talk about some of those technologies. One of them is a company called Nacuity that is developing an antioxidant.

They are in early Phase 1 testing, but they should be in Phase 2 in the next 12 months or so in retinitis pigmentosa. Then there is rod derived variability factor to hopefully protect central vision with retinitis pigmentosa. If you have an opportunity to get a genetic test that would be a good place to start.

Jason Menzo: To echo Ben's point about the importance of genetic testing, which is why with our My Retina Tracker we offer the free genetic testing program, because many of the clinical trials on the horizon are dependent on knowing individual's genetic mutation so that the appropriate folks are enrolled in the trial. We encourage anyone who is interested in learning more to check out fightingblindness.org or send an email to info@fightingblindness.org. The importance can't be understated knowing the individual's genetic mutation. Chris, I know you said a few times folks who have only used the dial in by phone method to access this call could send questions to info@fightingblindness.org to ask a question during the call. Have we had any questions come in that we should address?

Chris Adams: Yes. One of the questions is when we were talking about the RD Fund, can we explain again the five projects that are currently being funded by the RD Fund.

Dr. Ben Yerxa: Yes. I can do that at a high level and on the next call we'll get more meaty updates. I just mentioned one company, called Nacuity that is in essentially Phase 1 with an oral formulation of an antioxidant and they should be in patient trials either at the end of this year or early next year. We're waiting for some important updates over the next few months on that and hopefully at the next conference call we can give an update. Gene therapy for rod derived cone viability factor. That should be in the clinical for late 2020 for retinitis pigmentosa. We also made an investment in a public company called ProQR. They gave a clinical program for Usher Syndrome 2A and are actively enrolling in this program right now. Hopefully there will be interim results about a year from now depending on the speed of their enrollment. So, we are waiting for results on that. Recently we invested in a company with technology

that came out of Dr. Tom Reh's lab at the University of Washington called, Nayan. This is a known investment. There hasn't been a big public announcement about it, but essentially those technologies that Tom developed by studying some of the biological regenerative processes that amphibians like salamanders have. They have that approach, but this technology is still a ways away. The last one we made an investment in has not been publicly announced but it should be in the next couple of months. We'll give you an update on that. I'll tease you with the category - it's in the area of optogenetics.

Jason Menzo: Specific to information around the RD Fund, we have a great website called RetinalDegenerationFund.org that we do push updates related to portfolio companies that are making milestones. Future investments we may make will be certainly announced there. I would say it's probably pretty fair to say the profile in terms of size of investment and structure of investment that we've made historically through the Retinal Degeneration Fund should serve as a pretty good model of what you can expect from the RD Fund as well. In many cases, we're coinvesting with other entities and so there's sometimes issues with confidentiality because we can't get maybe as deep into some of the answers to questions that folks may have surrounding confidentiality but certainly anything that can be shared publicly we do That's where we push the latest information and updates that are publicly available on portfolio companies within the RD Fund. Any other questions, Chris that have been emailed in?

Chris Adams: Yes. We received one question which is what is the data on the rejection of stem cells after a year or so of stem cell therapy for RP and also does the stem cell treatment preclude later gene therapy?

Dr. Ben Yerxa: Yeah, kind of a tough question. This is Ben Yerxa. When it comes to the stem cell therapies, especially the ones being sponsored by industry, we have to wait for them to make announcements on their data. There are different approaches with stem cells. Some are related to putting in cells that release neuroprotective factors and others are meant to regenerate by directly repopulating those cells. I don't have a very specific answer

except that we track these efforts and try to understand their data as they emerge. There are a couple in Phase 2 but in small numbers of patients, so I think we have to let them do the work and report the data and we'll know in due course in the next, 12 to 24 months how those approaches are going.

Jason Menzo: Very good, thank you, Ben. We have about seven minutes left in our time together today. Chris, are there any other questions that have been emailed in?

Chris Adams: We have a question that came in in the Q and A box. Have there been any updates in the RLBP1 trial in Sweden?

Dr. Ben Yerxa: That's not something I'm aware of personally, this is Ben Yerxa. Ben Shaberman, maybe you could chime in.

Ben Shaberman: We are not aware of any updates. We posted an article on that trial right after the ARVO meeting, our big global research meeting, in Vancouver last May. So that trial is still at an early stage. To my knowledge they have not reported any results. That's for people with mutations in the gene RLBP1 that cause RP. That's a Novartis trial.

Jason Menzo: Thank you, Ben. All right. Looking at one more question, there's a question that came in, can you give us an update on clinical trials regarding the Stargardt disease.

Ben Shaberman, can you give a high-level overview of the current status of clinical programs in Stargardt.

Ben Shaberman: Sure, there are actually a lot of therapies in the clinical pipeline for Stargardt disease or about to enter clinical trials, The biotech company Alkeus is conducting a multi-center Phase 2 clinical trial for a drug that targets the toxic build-up in the retina that is thought to cause degeneration and vision loss. The emerging therapy is a modified form of vitamin A that burns cleaner than the natural form so there’s less accumulation. The StarGen gene therapy clinical trial is underway in Oregon and Paris. That's being conducted by Sanofi. The treatment replaces mutated copies of the gene

ABCA4 with normal copies. They have not reported results recently as that is ongoing. There's another molecule soon to enter a clinical trial if it hasn't already, related to a retinal binding protein. And that too is targeting the accumulation of waste products in Stargardt disease. There is an emerging gene therapy using the adeno associated virus for ABCA4 which is the gene associated with Stargardt disease. That's moving toward a clinical trial. It isn't in a clinical trial yet. Those are some of the things I know of off the top of my head. There are more. As you said earlier if you want to submit a question in to info@fightingblindness.org, I can give you even more information.

Jason Menzo: Excellent. Thank you, Ben. I'm looking at the clock. We've basically come to the end of our time. I want to take this opportunity to thank everyone for dialing in today, for participating on the call, in particular Congressman Sessions, if we were all in a room together, I think you would be getting a standing ovation for not only your comments today but like I said earlier, the long standing support for our cause and the Foundation Fighting Blindness and for you to carve out time to be part of this call means a lot to us and we thank you very much. For those who have questions that you didn't ask today or that we didn't have an opportunity to answer on the call, please send an email to info@fightingblindness.org and we will make an attempt to answer each question in the coming weeks. The recording of this call will be available on our website in the next couple of weeks along with the transcript. I want to thank everyone for participating today and we will be announcing the next *Insights Quarterly Forum* in just a few months. We're going to do this every quarter and we really appreciate you carving out time out of your day to join us and wish you a great rest of the day. Thank you.