

**Foundation Fighting Blindness**

***Insights Forum Call Transcript***

**March 21, 2019**

JASON MENZO: Good morning. 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 second 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 provide an operational and financial review, and our Executive Vice President of Research, and Interim Chief Scientific Officer, Dr. Brian Mansfield, will share a research update.

There will also be a question-and-answer period at the end of the call. 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. I'd now like to turn the call over to our CEO, Dr. Ben Yerxa.

DR. BEN YERXA: Thank you, Jason. Good morning 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. We are the catalyst in funding breakthrough research and innovative science that provides preventions, treatments, and cures as fast as possible.

I'd like to briefly highlight some of the exciting recent developments both at the Foundation and within the broader community. We recently brought together stakeholders and supporters for the 2019 Investing in Cures Summit, which was held March 1 & 2 in Raleigh, North Carolina. The Foundation hosted a cross section of industry partners, investors, researchers and other members of the IRD community. In total, there were 165 attendees and 18 presentation and panel sessions on the latest advancements in clinical trials and industry

partnerships.

The Summit featured presenters from around the world, including translational research experts, clinical trial investigators, and the companies that are poised to take emerging therapies across the finish line. The Investing in Cures Summit was made possible thanks to generous support from our Seed Partners, Eloxx Pharmaceuticals and MeiraGTx. Based on the success of this meeting, the Foundation is planning on hosting this event annually.

One of our major initiatives in 2019 is to continue expanding translational and clinical research projects for treatments and cures through our retinal degeneration fund, or RD Fund. Launched in late 2018, 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. To date, four investments have been made: ProQR, SparingVision, Nacuity, and most recently in Nayan Therapeutics – totaling \$25M in currently committed capital.

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 Gund Harrington Scholar 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, which is developing antisense oligonucleotides (AONs) for several ophthalmology programs, recently released interim data from its lead ocular program, CEP290 (LCA10), showing promising, preliminary data for improvements in both structure and function in a Phase 1/2 clinical trial. The program for Ush2A that FFB is co-funding with ProQR has started enrollment in a Phase 2a clinical trial, with initial clinical proof-of-concept towards the end of 2019. ProQR recently announced programs in Stargardt disease, and for undisclosed mutations in Usher and LCA.

SparingVision is developing a gene therapy program to deliver rod-derived cone viability factor (RdCVF) to patients with retinitis pigmentosa. SparingVision recently completed a manufacturing contract in the U.S. for

production of cGMP vector with plans to enter the clinic in 2020.

Nacuity continues to make progress towards its planned Phase 2 clinical study in RP patients. As a prerequisite, Nacuity is conducting a second safety and pharmacokinetic study in Australia with its optimized tablet formulation, which is expected to begin soon.

It is truly encouraging and exciting to see the progress these companies have been making. The RD Fund is generating significant interest, with many exciting investment opportunities for companies and programs that can be in the clinic rapidly. We look forward to updating you on new and existing RD Fund investments and their advancement over the coming months.

The pace of change in our field continues to accelerate. The recently announced acquisitions of Spark Therapeutics by Roche and Nightstar by Biogen signal the high level of interest and optimism for validated gene therapy delivery platforms.

Johnson & Johnson's Janssen division signed a collaboration and licensing deal with MeiraGTx for its inherited retinal disease portfolio, including candidates for achromatopsia caused by mutations in either CNGB3 or CNGA3, as well as X-linked retinitis pigmentosa. Also, Sanofi Genzyme recently announced that as part of a company-wide portfolio review, it intends to seek a licensing partner for their Stargardt disease and Usher's Syndrome type 1b programs.

Overall, these developments are a sign of the evolution and maturation of gene therapy as a promising modular therapy, especially in eye diseases.

Shifting back to the Foundation, we continue to enhance our team to define strategies and initiatives to propel us to new heights as we accelerate progress towards our mission. We recently added Dr. Todd Durham as vice president of clinical & outcomes research, Chris Adams as vice president of digital marketing and communications and Michelle Glaze as associate director of professional outreach. Todd is focused on clinical research, such as natural history studies and development of novel endpoints for IRDs, Chris is leading our team on branding and external communications, and Michelle will be working with Ben Shaberman, who is taking on a new role as well, focusing on scientific outreach and community engagement. Jason will share more on this initiative in his remarks.

In addition, we are making a transition on our senior science team. After 14 years of valuable service, Dr. Steve Rose, our chief scientific officer, will be moving to an advisory kind of position. We are grateful for Steve's tremendous contributions to the Foundation and the IRD community and we look forward to continuing to work with him in his new role as senior scientific advisor. We are very fortunate to have Dr. Brian Mansfield, who also has significant expertise in this field and many years of experience with the Foundation, who was recently promoted to executive vice president of research and interim chief scientific officer.

As we continue to evolve the organization and prepare for future success, we have undertaken a 5 year strategic planning process. The key components of the plan include 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. This work will take some time to complete and we will report out on the high level plan likely in the fall timeframe.

To conclude my update, I'd like to share a "Mission Moment" - an inspirational story that gets to the heart of the Foundation's mission. Last fall, Foundation volunteers and staff hosted a small informational meeting in Rogers, Arkansas. There, we discussed the Foundation, and the research and services like genetic testing through our My Retina Tracker registry. One attendee, James, inquired about genetic testing for his wife and her sister. So, our Foundation team followed up and provided him with information on regional doctors participating in the testing program.

Now, fast forward to last month. James's wife was genetically tested and they learned she has mutations in exon 13 of the USH2A gene, which is the same exact mutation being studied by our partner ProQR and may qualify her for the clinical trial that just started. Finding trial participants is always critical for moving this therapy forward.

This is a story in which many elements came together - a passionate volunteer leader, a chapter event, My Retina Tracker and genetic testing, and an emerging therapy funded by the Foundation— that could potentially provide great benefits. This collaboration is the Foundation fulfilling its mission in a powerful, comprehensive way. Every Foundation event, donor, genetic test, and My Retina Tracker registrant is critical to our mission. And, more of these stories are happening thanks to the advancement of the research and our programs.

I'd now like to turn the call over to Jason Menzo, our Chief Operating Officer, so he can provide a quick review of the various operational initiatives we have under way to leverage the Foundation's resources and support.

Jason.

JASON MENZO: Thank you, Ben. I'd now like to provide an update on some of our key operational initiatives. Over the past several months, our team has worked hard to continue to evolve the organization to become as efficient and focused on our mission as possible. In that time, we have made multiple system and structural enhancements to the backbone infrastructure of the organization allow our people to step away from the administration, and let them focus on their research passions, outreach and advocacy and community engagement efforts that drew them to our mission in the first place. We are not "done" with these process improvements yet, but are making progress. Many of these enhancements are behind the scenes, while others will be more obvious, like our new online fundraising platform, called Classy, that we will be launching later this summer. In all cases, our goal is to improve the experience of our constituents, and free up our staff to do the important work of driving our mission.

One of the more obvious evolutions in the past few months occurred in January when we launched our new "Beacon of Light" logo and messaging. The image emphasizes our mission by putting the stacked words "Fighting Blindness" front and center, and stacks the word "Foundation" vertically to the left of the image. The real message of our logo however is the treatment of the spotlight shining through the words, flowing through "Fighting Blindness" from left to right. The intent is to convey how the Foundation projects a beacon of light, and hope, for those in our community, or who are newly diagnosed with an IRD. The Foundation sheds light on innovative research for treatments and cures illuminating a future where we've helped to bring light to darkness.

With our new logo and branding in place, we are also very excited to be launching an all-new Foundation Fighting Blindness website – [www.FightingBlindness.org](http://www.FightingBlindness.org), so stay tuned for more communications in the coming weeks!

As Ben mentioned, we have created a new focus on scientific outreach and community engagement under the leadership of Ben Shaberman, who writes our Eye on the Cure blog. This team is tasked with developing and executing our strategy to strengthen relationships with the eyecare physician community. Also, we are constructing a comprehensive "customer" experience for newly diagnosed patients when they and their families

are first introduced to the Foundation. We want to shorten the amount of time between someone being newly diagnosed, and them finding the Foundation. Then, when they find us, we want to usher them into our community with information and resources and encourage them to connect with our chapters, enroll in My Retina Tracker and join the fight through our various activities and initiatives.

Our community of volunteers, affected families and staff continue to raise awareness and funds for our mission. Sixteen VisionWalk events were held in the fall of 2018, with combined funds raised exceeding \$2 million. We also hosted several special events including the Denver Scramble for Sight, the Atlanta Golf Classic, the New York Taste for Sight, Visions Ball, Dancing in the Dark, Los Angeles Dinner and St. Louis Dining in the Dark. Collectively, these events raised more than \$1.5 million. In 2019, we have thirteen dinner galas, wine events and small events that will take place this spring, with combined revenue targets of more than \$2 million.

In terms of public health and education programs, we recently held Vision Seminars in Chicago, Raleigh, and Houston providing research updates and education on living with IRDs to hundreds of attendees. This spring, we will be hosting more than 35 research presentations across the country and will be hosting science calls for our members led by Ben Shaberman.

In addition, the Foundation and the Casey Eye Institute at Oregon Health & Science University will co-host the sixth annual Retinal Cell and Gene Therapy Innovation Summit in Vancouver, British Columbia, on Friday, April 26, 2019. The summit takes place just prior to the 2019 annual meeting of the Association for Research in Vision and Ophthalmology, or ARVO. At the Summit, representatives from biotech and pharma industries will come together with members of the physician and scientist communities to discuss rapidly emerging ocular gene, cell and novel therapies and strategize how to move the most advanced retinal disease therapy options forward.

A key part of our Five-Year Strategic Plan is expanding research funding. We were pleased to announce earlier this year The Free Family AMD Research Program, which is providing funding for 10 research projects over five years for the development of age-related macular degeneration (AMD) therapies. With an anchor investment from Dr. James Free and his wife, Carole, along with matching funds from the Foundation Fighting Blindness, this exciting program will begin in July 2019. In addition to the new research being made possible by The Free Family AMD Research Program, the Foundation is also funding an additional \$3.5 million for 11 other AMD research projects.

Finally, I would like to conclude my remarks with a brief summary of our financial position. As a reminder, the Foundation operates on a fiscal year that runs from July to June. As of December 31, 2018, our actual Fiscal Year 2019 unrestricted revenue was approximately \$10 million and expenses were \$6.2 million. We remain on track to achieve our budget for fiscal year 2019, which includes target revenue of \$24 million with more than 70% targeted to go towards research funding and public health and education.

We are very grateful for the generous support of all of our donors who continue to support the Foundation each year. This generosity has enabled the Foundation and the RD Fund to invest in cutting-edge science directed towards a variety of promising research opportunities.

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: 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 morning, I'm going to provide a snapshot of how far we have come in inherited retinal disease research, provide a summary of related clinical programs and conclude with some of the additional Foundation initiatives underway to accelerate this progress.

First, I would like to give a summary of the inherited disease landscape. Forty-eight years ago, when the Foundation first started (as the Retinitis Pigmentosa Foundation) very little was known or understood about inherited retinal diseases. We didn't know they were a group of several different diseases, we didn't know the mechanism of disease, we didn't know the genes that caused the disease, we had no treatments for the disease and we had no cures. Patients were told to go home, learn braille and prepare to be blind.

Now fast forward to the dramatic research environment and life-changing prospects we have before us today. We have a much better understanding of these genetically driven diseases, but we've also realized how complicated and diverse they are, with many sub-types stemming from defects in over 270 genes.

We have come a long way since those early days. Today there are two approved gene therapies available for patients - Spark's gene therapy for diseases caused by mutations in the RPE65 gene – called Luxturna, and in the

UK, MeiraGTx's gene therapy for AIPL1. In addition, there are several prosthetic devices for people who have lost most of their vision including Second Sight's Argus II Prosthesis, in the US and in Europe Pixium Vision's IRIS II Prosthesis and Retina Implant's Alpha IMS Prosthesis. While these are exciting advances, we still have a long way to go. So let me tell you about the therapies that are currently in clinical trials.

As we look across the clinical trial landscape, there are 7 key areas of tremendous progress. I'll highlight each of those briefly.

1. The first technology I will talk about is Gene Augmentation – If a person with a retinal disease still has viable retinal cells, and the gene causing the disease is known, then, there is the opportunity to correct the defect and restore function, in many cases, by adding back a good copy of the gene that is mutated. This is how the RPE65 gene therapy LUXTURNA works. With the success of Luxturna, we are seeing a lot of interest and industry competition in gene augmentation therapy. This is because the eye is a good target for gene therapy – it is small, requires very little of the expensive drug to treat it, if injected directly into the eye, you can easily look right into the eye and see what's happening inside the eye. So companies are attracted to test their gene therapy technology in the eye, creating competition, which is good for patients! Today, there are currently 23 gene-based clinical trials targeted at 13 different genes in trials undertaken by companies like AGTC, Nightstar, MeiraGTx, Spark, GenSight, Sanofi, Novartis, STZ Eye Trial, and Horama. Moreover, there are more than 75 different gene therapies in the preclinical pipeline. Several of these programs benefited from early preclinical funding support from the Foundation.

2. A second type of gene therapy is Optogenetic Gene Therapy – If the cells in the retina that respond to light are no longer working, then you need to take a different approach. Researchers are working on inserting light-sensitive genes found in bacteria into the human retina to restore light sensitivity. Two of these approaches are already in clinical trials – one program by Allergan and one by Gensight Biologics. Both of these programs benefited from early preclinical funding support from the Foundation. There are at least 16 similar approaches also in preclinical development.

3. A third genetic technology is Gene Patch technology – In some retinal diseases, it is not the gene itself that is mutated, but the instructions that tell the gene how to work. Gene patch technology is a way to cover up the mutation giving the wrong instructions. Today, there is one clinical trial in process targeting a particular mutation in the gene CEP290 that leads to a form of Lebers congenital amaurosis - LCA10. The initial clinical data

has indicated that the treatment is well tolerated and can result in improved visual acuity. The sponsor of this program, ProQR, is planning a pivotal Phase 2/3 trial that should start soon.

4. Yet another type of genetic technology is Gene Editing – This approach uses CRISPR/Cas9 technology. CRISPR/Cas9 can be thought of as molecular scissors that can be guided to a specific mutation in a cell, which enables a therapy to cut out the mutation and then paste in the corrected piece, which then allows the gene to function normally. Today, there is a pipeline of preclinical work using this very precise and innovative approach, and the company Editas has announced it has FDA permission to start a clinical trial for a particular mutation in the gene CEP290 that leads to a form of Lebers congenital amaurosis - LCA10. And again, there is a rich preclinical pipeline in development at a number of companies and academic research institutions.

5. There is another emerging technology called RNA-Nonsense Mutation Targeting – A gene is like a master template. When a gene makes its protein, it does so by making a lot of copies of the master template – this is RNA. So another approach to overcome a mutation is to target the RNA copy of the gene. There are some small molecules in development that can camouflage a particular type of mutation called a non-sense mutation. A non-sense mutation is like a road block in a gene – it stops the whole protein from being made. By camouflaging the mutation, the roadblock is removed from sight and the whole protein can be made. Since non-sense mutations are found in most genes, this approach is not gene specific, but could represent pan-disease therapy, that could address multiple causes of inherited retinal diseases (IRDs), not just one specific gene defect. This technology is in the clinic but not yet for retinal disease, but companies, including Eloxx Therapeutics are developing retinal programs to bring this therapy to the clinic for the inherited retinal diseases (IRD).

6. Now let me talk about cell therapy. Cell Therapy is very different from gene therapy – when retinal cells have been lost, gene therapy will not work because the cells you want to target are gone. So an alternative therapy is to grow new retinal cells in the lab and implant them in the eye to replace the lost cells. There are different types of stem cells that can be used for this, derived from eye donors, embryos, skin and blood. These stem cells can be grown in the lab, guided to turn into different types of cells – like photoreceptors or RPE cells and then transplanted back to replace the missing cell type in the retina. Today, there are more than 20 clinical trials underway, many initially focused on AMD, but notably programs by J-Cyte, ReNeuron, and Astellas, are targeting retinitis pigmentosa and Stargardt disease. To date they appear safe and J-Cyte have reported a trend in improvement in best corrected visual acuity (BCVA) in the treated eyes compared to untreated eyes. Again, the Foundation has actively funded several of these programs during preclinical development.

7. The final therapeutic approach I will mention is Small Molecule Drug Therapy – small molecule drugs are the traditional drugs you will be aware of – synthetic chemicals like aspirin and statins. These treatments are usually not targeted at a specific gene but rather at biochemical pathways that are disrupted by a gene mutation. So they could also represent potential pan-disease approaches. Today, there are more than 8 compounds in clinical development for either Stargardt disease or retinitis pigmentosa by Nacuity, Nayan, - both of which the Foundation has supported - as well as by companies such as Mireca Medicines, QLT, Alkeus, Lin Biosciences, Acucela, Ophthotech and Ophthalmos. And there is a very promising preclinical pipeline.

One of the key requirements to moving these exciting clinical programs forward is the ability to find the right patients to enroll in the clinical trials. My Retina Tracker is a global patient registry for inherited retinal diseases, managed by the Foundation.

My Retina Tracker is designed with state-of-the-art database technology to protect patient privacy. Data in the registry is available to qualified researchers who have applied to the Foundation through a rigorous scientific review process for access to the data for research or to help accelerate clinical trial enrollments.

Since many of the current clinical trials focus on gene-specific therapies, it is important to know the genetic basis of disease for each person. To overcome the barriers to genetic testing, the Foundation has, through the My Retina Tracker registry offered genetic testing to members of the registry since late 2017. To date, over 4,300 samples have been submitted for analysis and over 3,400 reports have been returned to patients.

The total size of the registry now includes 22,000 participants, with approximately 11,000 detailed profiles and 1/3 of those individuals having been genetically tested. Our goal over the next five years is to have 40,000 registry members with more than half, or 20,000, with genotype information available. Since genetic testing is not typically covered by insurance, the Foundation has negotiated low rates with testing providers to encourage patients to complete the analysis. This is an area of high need for funding and participation.

Two years ago, the Foundation established the Clinical Consortium, which includes 20 clinical centers of excellence with significant experience in inherited retinal diseases (IRDs) and with standardized assessment protocols. The readiness and expertise of these centers helps speed up the often lengthy and expensive process of setting up and conducting clinical studies.

Currently the Clinical Consortium is conducting natural history studies, which help us understand how a disease progresses and how variable that progression is between affected people. The Foundation recently completed the ProgSTAR natural history study of Stargardt disease, which has been of wide interest to researchers and industry and the consortium has initiated the RUSH2A study looking at the natural history of diseases caused by mutations in the gene USH2A.

In addition to the natural history studies, the Clinical Consortium is in the planning stages for three additional types of projects to be initiated as part of our Five-Year Strategic Plan. These include (1) the development of a patient reported outcome questionnaire for adults with non-syndromic RP, which will be designed to measure health-related quality of life within the context of clinical trials; (2) the development of “disease progression models” using advanced statistical methodology, which will enable optimal study designs for clinical trials and improve their chances of success; and (3) opportunities for affected individuals to participate in qualitative research. We believe these initiatives will have numerous benefits for the Foundation, the RD fund, and our stakeholders.

In summary, we are proud that the Foundation Fighting Blindness has raised and invested more than \$750 million to battle retinal diseases through scientific innovation. Together, we are on track to make greater strides in 2019 and make a meaningful difference in our drive to end blindness.

We’d now like open the call to take your questions and comments. Operator, please provide the instructions for asking questions.

OPERATOR: As a reminder, to ask questions you may continue to type questions into the Q&A panel, click the button at the Zoom interface to submit questions and comments via text there. If I would like to join the conversation, and ask a question verbally we welcome that. You can please raise your hand at the bottom of the Zoom interface and click to raise your hand. We have a couple of hands that are up right now. Just in case that was unintentional, I'm going lower those hands. If you have any questions, that you would like to speak verbally with the group, please raise your hand now. Third, if you are on the telephone only and would like to submit a question, via email, please send emails to [info@fightingblindness.org](mailto:info@fightingblindness.org).

JASON MENZO: Thank you very much. We have a couple questions that were submitted in advance, so we'll start with those, then we'll go to the open lines. It's fun that we have so many different ways people can ask

questions. We've got chats and pre-submitted emails, then those with the hands raised. The first question I'll address to Brian was submitted in advance. The question is how do I get a list of institutions performing research and conducting clinical trials? Specifically, for my condition? And how do I get involved in clinical trials? Brian, if you could answer that?

DR. BRIAN MANSFIELD: Yes, thank you, that's a very common question we get. And I see that the questioner had actually had a genetic test so they understand the genetic cause of their disease but they don't actually mention it. My first comment would be, that I hope anyone who is in this position, has considered being in My Retina Tracker. If not, I would encourage them to join and share that particular information in their profile about the gene that is causing their disease. As I mentioned before, this is really important because, it helps us contact you if there's a specific research in your gene where you can help. The sort of thing you can help in, are companies want to know questions about what do you want out of the therapeutic. They will hold these groups called patient focus groups where they will talk to you about your disease, what living with your disease means to you and what are the most meaningful changes that therapy could bring about for your disease?

And also, of course if we're being asked to help identify people for clinical trials or natural history studies, that is another reason for being in My Retina Tracker if you have that information, please, that is a good place to put it.

For current clinical trials relevant to your gene, you can go to [clinicaltrials.gov](http://clinicaltrials.gov). Which is a web site -- you can type it into your web browser. And then you can use the search options on that site, to search for your disease or you can be more specific. You can put in your gene and see if there are any trials that are registered on the site. Now, I really have to warn everyone, to be careful how you use [clinicaltrials.gov](http://clinicaltrials.gov). It is a really valuable resource. It is very valuable to the entire clinical community to the entire research community. But, there's some warnings you have to be aware of. Not all trials listed on the site are approved by the FDA. And you only really want to consider participating in a trial, that has been approved by the FDA. Now when the FDA approves a trial, it approves what is called an IND, investigational new drug application. You should only consider trials which have an IND. There are 3 letter acronyms you'll hear around the clinical trial sites, IND is the one to focus on.

I would give people a particular warning if they're looking at cell therapy trials because this is where we find a number of trials or so called trials, which do not have FDA approval, which do not have an IND, so they will have a number of other 3 letter acronyms around them it is not IND. Please pay very close attention to that and also, remember, you should never pay for a clinical trial. If you're being asked to pay for a clinical trial, it is very unlikely, it is an FDA approved trial. You should probably not consider it.

Finally, when you use resources like Clinicaltrials.gov to find trials relevant to you, I strongly encourage you to go back and speak to your retinal health care provider, about that trial before you sign up. If you need some guidance to find a retinal care provider who has the expertise to guide you, you're always welcome to contact the Foundation and we'll help to connect you.

You can do this by just sending an email to [info@fightingblindness.org](mailto:info@fightingblindness.org). To find out other information about your diseases and genes, go to our web site [FightingBlindness.org](http://FightingBlindness.org) and look under the News and Research drop down menu where you will find a lot of information, blogs newsletters and other informational materials about current research, that is updated quite frequently.

JASON MENZO: That's great, thank you Brian. I mentioned earlier in the call that we are launching all new web site in the next couple of weeks. One of the enhancements similar to clinical trials.gov is that we'll have a pipeline including all the latest research advancements of trials that are happening in any of the disease spaces listed on the web site. That will be a nice resource for folks. There were several questions that had been emailed in advance and even sent in here today that were related to is specific research going onto find treatments for X. Rather than spending the time here on the call reviewing each individual program, we would refer people to the resources that we just mentioned.

DR. BRIAN MANSFIELD: Yes, again for people who have had a genetic test and are particularly focused on understanding what research may be available for their particular case, I would encourage them to send an email to [info@fightingblindness.org](mailto:info@fightingblindness.org) mention they had a genetic test. If you're in My Retina Tracker, go into the Tracker and based on what gene mutation you have, there will be carefully crafted information specifically, relevant to you.

If you're not in My Retina Tracker, then let me know that. We can find another way that you can send that information to us in a secure manner, so we can give you very specific information.

JASON MENZO: We have another series of questions about My Retina Tracker, specifically the genetic testing component. Can you speak to how would an individual get enrolled, and specifically, whether or not we cover the cost of genetic testing and how that process would work at a high level.

DR. BRIAN MANSFIELD: My Retina Tracker is our registry. And there is genetic testing which will be at no cost to you, if you're a member of My Retina Tracker. Joining My Retina Tracker to get a genetic test is not a good decision. But if you are a person who is really focused on helping the Foundation advance research, development in these diseases and you would like to be contacted when there's a relevant industry or clinical interest in your disease of interest -- then, joining My Retina Tracker is a very good route to go. If you're a member of My Retina Tracker you can apply for free genetic testing.

It is a very comprehensive test. And, because that comprehensive test, also, creates a very comprehensive and complicated report, we require that people in our genetic testing program, undertake genetic counseling. This is where we put you in touch with a counselor who is highly experienced in genetics but also highly experienced in the clinical symptoms of the disease and can formulate all of that material, together to explain to you, what your genetic test result means and then connect you with further resources.

The genetic testing and the genetic counseling are at no cost for the participant. The Foundation has funding to cover those costs for a period of time. The only thing that we require is that as a member of that study, you add that genetic data, in the deidentified manner in your registry profile, so it can benefit the field. The genetic counselor will make sure it's entered correctly.

JASON MENZO: Excellent. Thank you. One more question for Brian and then we have a question for Ben.

For Brian, in November, the Foundation reported in the "I am the Cure" blog it was going to invest \$2.5 million in the search for the elusive retinal disease genes and mutations. Can you provide a status on the program and when that will be started?

DR. BRIAN MANSFIELD: That's a good question. The elusive genes program has actually been running for a number of years, funded by various awards and, as the "I am the Cure" blog said, this was recently renewed with a program project grant to doctors Gann, Iagarie and Pierce. The program is up and running and they are pushing through the analysis of these patient samples, using a wide range of technology. When you're trying to find an elusive gene you actually step beyond the DNA sequencing stage, which is the first step people use to try to find these genes and you step into looking at that molecule. I talked about RNA and also, the proteins that are being made so you take a very broad view of the whole biology of the gene, to try and find little bits of evidence in all of these different areas, which will allow you to draw a conclusion that identifies a new gene that causes

the disease. It's a really exciting program that takes a lot of resources. It is a very intense program, that takes time to pull these bits of data together. But it is well under way.

JASON MENZO: Very good. There was a question that came in during the presentation. Just for clarification, this presentation today did not have any slides. So those of you who are listening or are viewing the event with the closed captioning link that we sent, the reason for the web interface was purely for the Q&A session to increase the accessibility to allow closed captioning. There were no slides or any other materials as part of this presentation. It is purely an audio presentation.

We have a question that was submitted in advance that I'm going to send to Ben. The question is, the EYS gene is a potential contributor to autosomal recessive RP. Are there any planned studies related to this gene in the scientific community?

DR. BEN YERXA: Thanks Jason another great question. This is actually a topic that came up when we started working with our Clinical Consortium to discuss what should some of the next natural history studies be conducted in. And the EYS gene came up as one of the top candidates to consider. We're still working through the protocols and the ideas for potentially moving that forward as a stand alone natural history study. We're also looking at a more universal kind of protocol to bring in many different gene mutations to see if we can study them together as sort of a master cohort of sorts. But what is interesting about the EYS gene is that there's also significant interest from industries. We're trying to work out the game plan on the protocol while also getting both scientific and financial support from industry.

JASON MENZO: Ben, we have several questions picking up on the comments that you made in the presentation, about the active role that industry is playing in this category now. There are two questions related to any insight as to why Johnson and Johnson and subsidiary Janssen excluded the LC8 candidate from the partnership deal. The second question is any insight or additional comments on the Sanofi outlining of the programs. Any additional color or commentary from your perspective on those two transactions?

DR. BEN YERXA: I can add a little bit of color. These are big transactions. Regarding the Johnson and Johnson deal, we don't know why they chose some programs over others but the way I see that deal is, it's really like an acquisition of the company, in disease. They acquired a lot of the main assets but sometimes, the innovator wants to hold on to one or two assets for themselves to develop and take all the way so they can grow as a

company and bring on other programs. It's speculation but my guess is that they wanted to keep that for themselves because they like that one and wanted to keep one in-house.

Regarding Sanofi, you know big companies go through all kinds of changes. Especially when there's a change in leadership, which I think is really the case with them. We met with them yesterday. We met with their senior management teams in RD, to talk about their commitment to IRDs despite the fact they are, in the process of out-licensing those two programs, Staargardt and USH. They are still active for gene therapies for IRDs but they have not disclosed what those programs are. They did reaffirm the commitment to continue to do work in that space.

What we're doing at the Foundation regarding the out-licensing programs is just trying to be there, to help those programs find a good home. We're involved in the process to the extent we can be and we'll do our best to make sure they enter the hands of very competent individuals.

JASON MENZO: Very good thank you. Joshua, I know there are a hand full of folks that have raised hands to ask their question aloud. Let's go to that forum to answer questions from the audience out loud.

OPERATOR: Thank you so much. We'll start with Martin.

MARTIN: First of all, thank you very much for this presentation, and for all of the work that you do for us. I'm a 56 year old male. I am registered with My Retinal Tracker and my gene has been identified as USH2A. I've been following the RPE675 treatment, and, there's reports saying that people are having improved vision. Can someone speak to the kind of improvements that people are getting from something like that? I know it's not, for the USH2A, but it may give me an insight into what I can look forward to.

Is there a way of measuring that someone has moved from being blind to legally blind or legally blind to sighted? Can you make that simple for us to understand the progress? Thank you.

DR. BRIAN MANSFIELD: Yes, Martin, this is Brian. Perhaps it is useful to explain that one of the tests that was used during the clinical trial that was required to show that people could improve with the therapy. Because it is standard methods of measuring improvement in, retinal disease, over the past has been the improvement visual acuity or the improvement of the light sensitivity in the visual periphery. And those were

really not relevant to the RP65 disease where there was very little visual acuity initially. What they used was a mobility course in a dark room in which they have set up a winding path a little bit like a maze with various objects you have to step around or over or avoid in some way.

They varied in a controlled manner, the light level in the room, so they would start off in a very dark room and, they would have cameras photographing the people's movement, as they moved around the room. Of course they were stumbling because they could not see. As they raised the light level up, what they found was that the people would start to move with less mistakes, until they got to a light level where people could move without any mistake at all. They used this on people before they started the clinical trial and people after the clinical trial and what they could show was that the people treated were able to move around at a light level much lower than they had been prior to being treated.

Increasing the general sensitivity in low light situations improves an area of central vision with better visual acuity in general, so people are finding it's much easier to move about in their life, to recognize things, go about daily living. They may not be reading the "New York Times" in small print at the moment, but that gives you a sense of the general improvement that is being seen.

JASON MENZO: Very good, thank you Brian. Thank you for your question Martin. I know we have a couple other hands raised. I do want to make a comment on several questions that have come in with regard to housekeeping with this call.

The entire call is being recorded and it is also currently being closed captioned in real time for those who accessed that link. The transcript of the call will be posted to our web site and we will send out an e-blast once that is up to our whole community, not just those who participated. So be aware that in the coming weeks the entire transcript of this call will be available to revisit.

There were also several questions about which I'm personally excited. These include how to get involved from a community perspective. We do have a very active network across the country of chapters in 40 plus markets across the country with Vision Walks all over the country. We have many special events so anyone who is interested in becoming involved in any of our community outreach, or grassroots aspects of our organization, please send an email to [info@fightingblindness.org](mailto:info@fightingblindness.org). I'll get the emails sent to me and we'll distribute to those folks in the appropriate geographies. Thank you for those who are interested -- we love the energy of our grass

roots community. Joshua, let's go back to the lines see if there's anyone else that has a question?

OPERATOR: Next we'll go to Frank.

FRANK: My appreciation to everyone at the Foundation for working so hard to make our lives better.

I've been following the protocol of Dr. Berson's study in Boston with vitamin A palmitate, fish and lutein. I would like to though an update about the status of the scientific Efficacy of that approach.

DR. BRIAN MANSFIELD: Thank you Frank, we still believe that is a treatment that will assist in slowing the rate of progression. For RP we encourage people to do that to maintain their retina in the most healthy state it can be at the moment. We do have some studies that are looking at the people who participated in that initial study by Dr. Berson to see if we can learn any more detail about particular sub-groups of people who benefit more or less from it. That work is ongoing. They are basically analyzing the genetics of those people, to see if they can correlate any improvements with the particular genetic conditions.

JASON MENZO: We have a commitment that we will make to everyone who has sent in a question. If we don't get to your question here on the call, we will respond to you directly. Joshua, any other questions from the line? We will take one more question then we'll wrap up the call.

OPERATOR: We have a hand raised from Tina, we'll go ahead with her question.

TINA: Good afternoon. It is Tina Chu speaking. I'm calling from greater Boston. First congratulations to Brian for your new role. I want to extend my special thanks to Steve Rose for your support over the years and your leadership is appreciated. Congratulations for the great progress made. You've done a great job. My question is regarding Nayan, on stem cell reprogramming. Are you able to get multilayer for lamina sheath in photo receptor, if not, what's the distribution pattern of the newly formed cell, peripheral or more posterior and are what is the phenotype.

DR. BRIAN MANSFIELD: This is Brian, thank you for that question and thank you for your kind comments as well. Much appreciated. Your comment is probably a little too detailed to handle in the last minute or so we have here. I would be happy to talk to you more offline if you're interested, if you just send an email to [info@fightingblindness.org](mailto:info@fightingblindness.org), Tom Reh's technology is trying to deprogram, the rods, while leaving the cones, in

their differentiated form. It is considered that when rod cells are fully mature rods, they carry what is called a disease liability. This then impacts the cones which leads to the gradual loss of the functional vision. Dr. Rey's approach is try to persuade rod cells to turn off their rod like features and by doing so, remove that liability that they hold towards the cone cells, allowing the cells to survive successfully.

I hope that at least partially answers your question but I would be happy to share more with you through an email exchange or another phone call.

JASON MENZO: Very good. Thank you very much. I will say as we're wrapping up the call -- we did receive, many questions, about My Retina Tracker specifically and the genetic testing program, including questions about sites within the geography or the process. We will follow-up, over email with the detailed responses to each of those questions to be able to assist anyone interested enrolling in My Retina Tracker and/or the genetic testing program.

We would like to thank everyone for their time today.

There will be another Insights Forum call scheduled for next quarter. We really appreciate everyone's engagement throughout the call and with that I'll turn it over to Joshua to close the call.

OPERATOR: Thank you to all participants for participating in the webinar today for the Foundation Fighting Blindness Insights Forum. That concludes today's session, please enjoy the rest of your day.