

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

Chris Adams, Vice President, Marketing & Communications:

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

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

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

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

At this time, I would now like to hand the call over to Jason Menzo.

Jason Menzo, Chief Operating Officer:

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

I would like to welcome everyone to our quarterly *Insights Forum* web meeting. The purpose of this session is to update you on the latest developments here at the Foundation Fighting Blindness and within our broader community.

Here is our agenda for today.

- First, I'm going to be highlighting recent activities and events focused on increasing community engagement, fundraising and public awareness of our mission.
- I will then be handing over the call to our Executive Vice President of Corporate Development and Chief Business Officer, Peter Ginsberg, who will provide an update on corporate partnerships and a summary of our financial performance through March 31, 2021.
- Our CEO, Dr. Ben Yerxa, will then share an update on recent developments in the science, research and clinical landscape. We will then conclude with our featured guest speaker, Kari Branham, who is Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling at the University of Michigan's Kellogg Eye Center. Kari has been partnering with the Foundation for many years, and we are very excited to have her here today to talk about genetic testing for patients affected with inherited retinal diseases, and share why this testing is important, how it can be accomplished, what that process looks like and the importance of genetic counseling as part of the process.

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

We welcome your feedback or suggestions related to this webcast, or the Foundation in general. You can reach us anytime at the email address info@FightingBlindness.org.

I'd like to start the call by recognizing the incredible efforts of our entire Foundation Fighting Blindness community, including our volunteer Board of Directors, National Trustees, chapter leadership all over the country, our researchers, clinicians, partners in the corporate world and of course, our professional staff. It is safe to say, that the past year is unlike anything any of us could have predicted, yet our community has adapted and dealt with this unprecedented and unpredictable environment with energy and grace.

As a leadership team, we have continued to take a hard look at our operating model, staffing, and overarching strategy. Our mindset has been to exit the pandemic stronger than we entered it, as an organization, and as we start to see the light at the end of the tunnel, I am proud to report that we are well positioned for the future and I'm going to talk a little about that today.

Along those lines, our outreach efforts have evolved, for the better over this past year, and building off of our many virtual experiences that we've adapted to, we are excited to continue hosting new and innovative ways to connect and engage with you, our community in the years ahead.

I want to take a few minutes to highlight some of the recent events we have hosted.

- First, on Sunday, February 28th, in celebration of Rare Disease Day, we hosted a first of its kind. A totally virtual dinner gala - we called it Hope from Home: A United Night to Save Sight.
 - This was a fun evening- we had celebrity emcee, Saturday Night Live alum, Kevin Nealon, and we had a variety of comedy, inspirational speakers, and special musical performances.
 - This event was really special, not only because of the whole presentation and various voices and acts in our community that participated, but really the highlight of the evening was that we used it as an opportunity to present our highest research honor, the Llura Liggett Gund Award, to Dr. Jean Bennett. Dr. Bennett is Professor of Ophthalmology, Professor of Cell and Developmental Biology, and Director of the Center for Advanced Retinal and Ocular Therapeutics at the University of Pennsylvania Perelman School of Medicine.
 - Dr. Bennett has worked throughout her entire career to establish the scientific underpinnings which made it possible to test the first retinal gene therapy treatment for patients with a blinding retinal degeneration. This work culminated in 2017 with the U.S. FDA

approval of LUXTURNA, the first gene therapy for any inherited disease.

- We were very happy to recognize Dr. Bennett with this incredibly prestigious award. It was a special moment and everyone that was in attendance felt the passion that Dr. Bennett has for this field and that we have for her and her work.
 - I also want to thank our Chairs for this event, Dr. Alice Cohen and Dr. Jonathan Steinberg, and the amazing event committee, for putting together such a unique fundraiser and entertaining community event. This night alone raised over \$600,000 dollars toward our mission, and more importantly, gave our community, from all over the country, a chance to socialize and enjoy each other's company virtually. It was something that probably would have been hard to conceive a year and a half ago, but in the environment that we are now in, to be able to pivot, the fun and entertaining evening was really special.
- Another series of popular events here at the Foundation are our National Chapter Vision Seminars.
 - These are educational webinars that are hosted by various local Foundation Fighting Blindness chapters and are presented free of charge through the generous support of The Chatlos Foundation along with other partners.
 - In March, our Boston Chapter president, Martha Steele, hosted a webinar with a panel of world-renowned stem cell researchers who presented the latest in emerging stem-cell therapies for retinal degenerative diseases, including therapies in, or moving toward, the clinic.
 - The webinar was moderated by our own, Ben Shaberman, Senior Director of Scientific Outreach at the Foundation. Panelists included Dr. David Gamm, from the University of Wisconsin-Madison; Dr. Thomas Reh, from the University of Washington; and Dr. Michael Young, from Massachusetts Eye and Ear and Harvard Medical School.
 - It was a great event - we had over 1,500 participants from all over the country, really all over the world.
 - Chapter webinars like these are a great way for everyone in our community to learn about the latest research on a variety of topics.

Also, it's a chance to interact with and ask questions to some of the world's leading experts in our field. We have more chapter webinars scheduled and you can always check our website at fightingblindness.org to find out the schedule of events.

- Shifting gears, I want to share a few upcoming events, starting with our spring National Virtual Vision Walk, which is scheduled for Saturday, June 12th.
 - Just as we did last year in the spring and fall, we are converting twenty individual Vision Walks that we would typically host all over the country and we are going to merge them into one national event.
 - This year, our goal is to raise nearly \$2 million towards our mission.
 - Throughout the day, members of our community from all over the country will be taking important steps towards our mission.
 - We would love every single person, of the 300 plus who are tuned into this webinar here today and everyone who watches this on demand later, to participate in Vision Walk this spring.
 - You can learn more on how to register as an individual and your team at fightingblindness.org/visionwalk. Please sign up or go to the website to learn more.
- We have one more really special event that I want to highlight coming up this summer. Two of our dedicated board members, Evan Mittman and Jason Ferreira, who are Board members of the Foundation team from New York, are joining forces to host our next virtual gala on Thursday June 24th. This is picking up on the success we had at Hope from Home which I just described a few minutes ago. The event is called Night for Sight and is presented by our good friends at Two Blind Brothers.
 - We'll be celebrating 50 years of fighting blindness with a moving and entertaining program and here's the special thing. Not only do we have fantastic stories and focus on our mission, but we are going to be joined by American music icon and nine-time Grammy winner Sheryl Crow – yes... that Sheryl Crow. To find more information about the event or to register, you can visit FightingBlindness.org/NightForSight.

Now I'd like to talk a little more about another major initiative we are undertaking. This is something really important to consider.

- Today, we believe that beating each inherited retinal disease is a matter of time and money – the science is ready. What we are lacking is the time and money to move that science forward. While that is encouraging, we're now encountering a new obstacle—the science is outpacing the funding, despite the significant funds that we've raised over the past 50 years.
- That's why we are embarking in a new campaign that we are calling Victory for Vision, a comprehensive, multi-tiered giving campaign designed to raise needed funds to pursue our mission, accelerate our research momentum and propel us into the next half-century, or however long it takes for us to meet our mission.
- The total fundraising goal for this campaign is \$50 million above and beyond our normal annual fundraising efforts. Without this additional funding, we are at risk of needing to slow down our research by as much as 50% in the years ahead. So this campaign is very timely and very important for our continuing to accelerate our mission.
- We are honored to have Marsha Link, Robert Heidenberg and David Brint as co-chair for this important effort, along with many volunteers across the country.
- The Victory for Vision campaign, when fully realized, will allow us to continue to invest in the best research across all stages of development and accelerate our progress towards our mission.
- To learn more about the Victory for Vision campaign, please visit VictoryForVision.com for the latest updates and resources.

Finally, we have a really important topic that I want to spend a little time talking to you about. We recently announced an initiative that is related to our chapter program. We are reimagining and relaunching the chapter engagement program for the network of more than 40 volunteer-led chapters across the country.

- To appreciate how important this is, I want to take a step back. Fifty years ago, in 1971, Ben and Beverly Berman, along with Gordon and Lulie Gund, and several other committed families, co-founded the Foundation Fighting Blindness. In 1972, Lulie Gund created and became president of the Princeton, New Jersey Chapter. She oversaw this chapter for 48 years.

- The Chapter program is the backbone of the Foundation’s mission. Our chapters provide resources, education, and revenue in the fight against the entire spectrum of blinding retinal diseases.
- As you may know, Lulie passed away in March 2020, at the age of 79, but she lived an extraordinary, generous life, and her vision to connect with the blindness and visually impaired community through our chapters will continue on in her honor.
- As the Foundation enters its 50th year, we are recognizing Lulie’s many contributions by relaunching our chapter program in her honor with the name “Lulie’s Next Chapter for Light and Vision”.
- This new program will include all new elements, such as Lulie’s Leadership Training, which will bring together the best of Chapter leadership to participate in leadership training and team development seminars, and Lulie’s Light Award, which will annually recognize the work and impact of leadership from the Foundation’s Chapter network from across the country.
- This initiative will ensure that all of our Chapters, like Lulie, will always be there helping guide us, especially for this important initiative.

As I wrap up today, I can share with you that across our many meetings and events, like our *Insights Forum* today, we are energized, we are passionate, and the members of our community are all involved – we have got some new faces and others who have been with us for years.

I am so proud to be a part of the Fighting Blindness family. Our entire community has stepped up in a big way to keep the Foundation’s mission moving forward – so I want to thank you for all your hard work and dedication.

Now, I’d like to turn the call over to Peter Ginsberg, our EVP of Corporate Development and Chief Business Officer, for a financial update. Peter ...

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

Thank you, Jason.

First, I’d like to provide a brief summary of our financial position, and then I’ll share some recent corporate sponsor updates that we are quite excited about.

I am pleased to provide the following financial update.

- For the first nine months in fiscal 2021, so for the period ended March 31, our unrestricted revenue was approximately \$13.6 million against operating expenses of \$9.4 million.
- We are tracking to our overall budget plan for fiscal year 2021, which includes targeted revenue of \$21.2 million against operating expenses of \$14.5 million, with greater than 70% of those expenses going directly to mission related efforts. And very importantly, the Foundation expects to spend roughly \$20 million this fiscal year on research projects that we hope and believe will lead to preventions, treatments and cures for people affected by retinal degenerative diseases and that's our mission.

We continue to work closely with leading and emerging companies in our field also that provide financial support for these initiatives.

- These collaborations provide companies with an opportunity to engage with patients, clinicians, other companies and also experts in our field, thereby enabling us to fund more retinal disease research, with the goal of accelerating the development of therapeutics for inherited retinal diseases.
- We are really excited about some of the recent new sponsorships, and we expect to receive additional sponsorships in the coming months.
- I want to point out and thank our corporate sponsors for 2021 which currently include: AGTC, the Allergan Foundation, Apellis, Astellas, Biogen, Genentech, Janssen, MeiraGTx, Spark and Two Blind Brothers.
- We are adding new sponsors to those lists regularly and we are excited about continuing to grow this list and we have many conversations ongoing to enhance and grow our sponsorship base.
- I would also like to highlight a new sponsor for the Foundation, Iveric Bio. Iveric was a key sponsor for our recent virtual Investing in Cures Summit, and Ben is going to talk more about the ICS program in his upcoming remarks.
- Iveric's lead therapeutic is in Phase 3 for geographic atrophy and Phase 2 for Stargardt disease, and Iveric also has several gene therapies in preclinical development for a range of inherited retinal diseases. Thank you for sponsoring Foundation Fighting Blindness, Iveric.

In summary, we are grateful for the strong support of our individual donors, corporate sponsors and importantly our patient and clinical communities. I'd now like to turn the call over to our CEO, Dr. Ben Yerxa.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you, Peter. Good afternoon everyone and thank you for joining us on our quarterly update call. Today I'm going to share with you several updates on our scientific and research initiatives and then highlight some of the progress being made within the broader community related to clinical trials and potential treatments for various IRDs.

Remember the heart of our mission at the Foundation is driving the research that will provide preventions, treatments and cures across the entire spectrum of retinal degenerative diseases.

Some of you may have seen our news from earlier this week, that Dr. Claire Gelfman has joined us as our new chief scientific officer.

- In this position, Dr. Gelfman is responsible for leading the overarching scientific strategy for the Foundation, including the Foundation's early translational and pre-clinical research programs. She will also serve as the primary scientific contact with the Foundation's Scientific Advisory Board, the Research Oversight Committee, academic institutions, and government science agencies.
- Dr. Gelfman completed an undergraduate degree in biology from Washington University in St. Louis, a Ph.D. in biochemistry from the University of Texas at Austin and a postdoctoral fellowship with the University of California at Davis.
- She brings over twenty years of successful ophthalmic R&D experience, along with a proven ability to manage cross-functional teams of scientists and business professionals. Most recently, Dr. Gelfman served as vice president of pharmaceutical development at Adverum Biotechnologies.
- We are really fortunate to have Dr. Gelfman on board to lead some of the most significant initiatives the Foundation Fighting Blindness is spearheading.

Earlier this month, we announced the investment of \$5.5 million in seven new projects through our Translational Research Acceleration Program, or TRAP, awards.

- TRAP-funded grants support efforts and guide projects toward an IND submission, the key step before a human clinical trial can start.
- Here is a summary of those new grants:

- First, Dr. Bärb Rohrer, from the Medical University of South Carolina, is conducting an animal study of a gene therapy designed to selectively deliver a component of complement factor H to temper the overactive innate immune system in age-related macular degeneration (AMD). The approach is designed to mitigate retinal degeneration caused by the immune response, targeting the damage where it is most likely to occur.
- Dr. Paul Yang is evaluating the drug mycophenolate as a therapy for multiple forms of RP and related conditions. Already approved by the FDA for inflammatory conditions, mycophenolate has been shown to reduce the accumulation of a molecule called cyclic guanosine monophosphate, which is toxic to photoreceptors.
- Dr. Mahdi Farhan, from the Usher 3 Initiative, is completing pre-IND toxicity studies to advance a novel small-molecule therapy for USH3A into a Phase 1 clinical trial. The emerging drug works by stabilizing the misfolded USH3A protein (clarin-1) and enables it to better move to its target location in retinal cells and to potentially preserve structure and function.
- Dr. Tom Reh, from the University of Washington, is developing a process to enable the human retina to grow its own new photoreceptors. Thus far, he has had success in mice, thus the TRAP project is for evaluating the approach in larger animals.
- Dr. Stephen Tsang, from Columbia University, is developing a gene therapy to increase aerobic glycolysis – this is a process that generates energy -- in cone photoreceptors of those affected by RP. He believes the approach may preserve cones for RP patients and would do so independent of the mutated gene causing the disease.
- Dr. Rob Collin, from Radboud University, is developing antisense oligonucleotides (AON) – these are tiny pieces of nucleic acid – to mask splicing mutations in ABCA4, the gene associated with Stargardt disease. The AONs target mutations in RNA, the genetic messages used to build proteins that are necessary for a cell's health and proper functioning.
- Dr. Hendrik Scholl, from the Institute of Molecular and Clinical Ophthalmology in Basel, Switzerland, is developing an optogenetic

therapy to restore function to dormant cone photoreceptor cells for potentially a broad range of inherited retinal diseases. This effort will advance late-stage preclinical studies.

These TRAP awards are key to getting academic research moved towards and into the clinic.

The next topic I'm going to highlight is focusing on the intersection of science and business in the advancement of retinal disease therapies.

- In mid-April, we hosted our annual Investing in Cures Summit in a virtual format. The online event hosted by the Foundation included lab tours, researcher and executive interviews, and business and science stakeholder panels.
- The most compelling news coming out of the Summit was the sheer number of therapies for retinal degenerative diseases that are now in, or poised for, clinical trials and have gained commercial support. More than 40 emerging treatments are in human studies with dozens more at the clinical trial doorstep. That's incredible.
- The event opened with compelling patient perspectives on blindness and the hope that research is bringing. There were more than 25 Summit speakers who highlighted the exciting opportunities for new therapies but also the various challenges to getting to the finish line, such as funding and manufacturing.
- The Summit underscored the need to bring many resources and stakeholders together to get a therapy into and through a clinical trial.
- Look for an email coming out next week as the content from the Summit is made public to our broad community.

The Foundation also recently hosted workshops on IQCB1/NPHP5- and CRB1-associated retinal dystrophies. These workshops included scientific discussions on nonclinical disease models and endpoints and input from families with affected individuals. This information is being compiled and will be shared broadly in the near future.

Let me switch gears now and talk about the Foundation's My Retina Tracker Registry, which is a single, international, comprehensive source of de-identified information for researchers, biotechnology and pharmaceutical companies, about people with an IRD.

- With the increase in gene and mutation specific therapies entering the clinic, understanding the gene causing the disease in each patient has become more and more important.
- Using the data in My Retina Tracker Registry helps us understand how common each type of retinal disease is, how it impacts people's lives, how the disease progresses, the genes that cause the disease, and helps researchers and companies to efficiently find people who might be interested in participating in research studies and clinical trials.

We continue to expand participation in the Registry and the related open-access genetic testing program, increasing the number of Registry participants to about 17,500 members with about 9,000 of those members having a genetic test. If you're interested in this, go to the website and check out how you can participate.

With more than 43 IRD-related clinical trials in process, the pace of new data readouts and clinical findings is rapidly increasing. Here are some of the noteworthy ongoing studies and recent announcements:

- Alkeus Pharmaceuticals is in Phase 2 clinical testing of ALK-001, an orally-delivered compound to prevent the formation of toxic deposits found in the eyes of patients with Stargardt disease and age-related macular degeneration.
 - Alkeus expects to present new clinical data for the Stargardt disease trial on May 7th at the Association of Research and Vision (ARVO) Conference. Please pay attention to that coming up soon.
- Another company that is actively engaged in moving treatments from academic labs into clinical trials is Iveric Bio.
 - The company's lead product candidate is Zimura, a promising C5 complement inhibitor which is in clinical trials for Stargardt disease and dry age-related macular degeneration (AMD).
 - For dry AMD, Zimura, slowed disease progression (that is, the growth of the macular lesions) by 27% in a Phase 2b clinical trial and has advanced into Phase 3 testing.

- Iveric Bio is currently enrolling its ongoing Phase 2b clinical trial of Zimura for the treatment of autosomal recessive Stargardt disease.
- The company is also hoping to launch trials for RHO-based RP and Best disease later this year.
- ProQR has recently announced results for a Phase 2a trial for its USH2A AON therapy, showing improved visual acuity for 6 patients with advanced disease and improvements in perimetry for 8 patients with early-moderate disease. ProQR is now planning to advance this treatment into pivotal trials.
- Atsena Therapeutics recently announced Cohort 1 results from its ongoing LCA1-GUCY2D gene therapy trial in a recent publication showing initial positive trends in safety and efficacy. This is yet another example of the significant progress being made from the clinical trials currently underway.

I would encourage you to check our website at fightingblindness.org for frequent updates.

Today, we are really pleased to have a special guest speaker on our call. Kari Branham is a Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling at the University of Michigan's Kellogg Eye Center. Kari has more than 20 years of experience working in ophthalmic genetics at Kellogg, where she provides patient care to those affected with genetic eye diseases, serves as a co-investigator on several clinical trials for patients with inherited retinal disease, and conducts research to understand more about genetic basis for disease in patients with IRDs. We are pleased to have Kari here today to talk the importance of genetic testing and counseling. Kari, please go ahead.

Kari Branham, Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling, University of Michigan's Kellogg Eye Center

Thank you so much, and good afternoon everyone.

It's really great to hear from Dr. Yerxa about so many exciting developments in the field of inherited retinal diseases. One of the themes that you will notice

about this exciting science when you heard him talking is that so much of the research is targeted at specific genetic causes of disease. In fact, when listening to Dr. Yerxa, it may sound a little bit like alphabet soup with many different names of the genes. ABCA4, CRB1, RPGR, just to name a few. At the individual level, understanding the disease is essential for their clinical care and understanding this genetic cause of disease is accomplished through genetic testing. Many people may think, why do I need to worry about it, why would I consider doing genetic testing. There are couple of different reasons that we think about why it is important to have genetic testing done when you're affected by an inherited disease or other genetic condition. One of the reasons that we think about is to help understand what the risk to other family members may be. I have many patients that I talked with who ask what's the chance for my children to be affected? Is anyone else in the family going to have this condition? Where did this come from?

Through the genetic testing that is one of the things that we hopefully help hope to understand a little bit better. In some families it may be very obvious that you'll see multiple generations of the family that are affected with the condition. In other families there may just be one person in the family and that can be a little bit more difficult just from looking at the family history to understand exactly what the genetic cause is. Even clinicians who are very experienced with working with patients with inherited retinal conditions can't always tell exactly what the genetic cause of the disease is or what the inheritance for that condition is going to be. If we can identify the genetic cause, we can understand exactly what the risk to other people in the family is.

A second reason that we think about doing genetic testing is to help confirm the diagnosis. For some of the different inherited retinal diseases, the symptoms that a patient presents with can overlap with other inherited retinal diseases and so if we can understand exactly what the genetic cause is, sometimes, we can get information about prognosis about what to expect in the future.

Another reason, going along with confirming the diagnosis is in the majority of patients that are affected with inherited retinal diseases, they are only going to have ocular symptoms, so only the eyes are going to be affected. But, sometimes conditions such as retinitis pigmentosa or RP can be seen in other conditions where there can be other parts of the body may be affected. This might be something like Usher Syndrome or Bardet-Biedl where you will have other parts of the body, other health problems going on, so it's important to understand that.

The third reason we think about genetic testing and why it is important is that you want people to understand if there are any treatment trials you might be eligible for. Some of the treatment trials that are being developed are what we call gene dependent. It means that you have to know exactly what the genetic cause is in order to be eligible for a treatment or a clinical trial. This comes in, again, the alphabet soup with many different genetic causes of disease you need to know what the specific cause of disease is to understand if there is a treatment. This would be like LUXTURNA, the FDA approved therapy with patients with retinal degenerations due to RP65 gene. There are several other gene therapy treatments in the clinical trial phase and being developed.

How is genetic testing done? It is something you would work with your healthcare professional. This may be your ophthalmologist; many ophthalmologists will order genetic testing for patients. Sometimes not all ophthalmologists are familiar so maybe visiting a genetic clinic or counselor to help arrange for that testing.

At the simplest level how the testing is done is basically just getting a sample of DNA, most commonly that can be done from a sample of blood or saliva. When the sample is sent to the genetic testing lab, the genetic testing lab will compare the DNA or genetic information from a patient who is affected with an inherited retinal disease with what we call a normal, or reference, basically a control sequence of DNA from people who do not have any eye conditions. Basically, they are looking for any differences between the patient sample and that normal reference control DNA to understand what the differences are.

What the lab does, is they will classify any differences that they have found as either disease causing or not disease causing. If it is disease causing, they would typically call it a mutation or a pathogenic variant. If we think back traditionally as I have been introduced as someone who has been working in this field a number of years, many years ago, when we started doing genetic testing, we started in research labs only. More recently there are more commercial testing labs where either the patient or insurance are responsible. Sometimes this testing might be quite expensive if insurance doesn't cover it. That sort of testing is still available but we are very fortunate now that there are sponsored research programs out there, such as the FFB open access program which can provide genetic testing at no cost to patients affected with inherited retinal diseases. You can visit the Foundation Fighting Blindness website for more information about the open access program.

One of the things I hear from a lot of patients, as well, especially ones that may be we saw 10 or 15 years ago, do they need to get genetic testing done again? If we think about the way genetic testing was done many years ago, at that time, it was really testing for maybe just a single gene or a small number of genes. Now, it is really the time to try again. We have learned a lot more about the number of genes that are responsible for disease and different ways of looking at these genes. Now the panel includes hundreds of genes.

So what happens, how do you get the results back? What happens after the genetic testing is done? The result of the genetic testing is provided to the healthcare provider that ordered the test. Hopefully what happens after these results are available is there will be a discussion with the patient about things such as what exactly is the cause of disease, did we clearly identify the cause of disease, what's the risk to other members of the family. Sometimes these can be very low, maybe a 1% chance or as high as 50%. Really understanding exactly what the genetic cause of disease can help that information. As I mentioned previously, sometimes there will be extra ocular symptoms that is in a minority of patients but something to be aware of if that is necessary for them.

Finally, we let the patient know about the status of the clinical trials. There are some retina specialists, some IRD specialists, ordering genetic testing who are familiar with the information but not all of them are necessarily. It is especially helpful to talk to genetic counselor. That's the job I have. There are many other genetic counselors out there. They are healthcare professionals who are specifically trained to talk to people about genetic conditions and really help them to understand this information. In fact, all the patients who are doing genetic testing through FFB have access to genetic counselors. I encourage you to talk to them, get that information that can be helpful for understanding your condition.

When genetic testing is done, basically, around 60% of the time we are able to identify a clear cut cause of disease. The remainder of the time these results may be negative or inconclusive. Negative means no genetic changes were identified that are believed to be the cause of disease or inconclusive means something was found but we're not exactly sure if that is something that is the cause of disease. Sometimes after the initial result it is still necessary to do additional testing from looking at other family numbers or things like that. Sometimes affected or unaffected family members or parents to try to help understand that information. Even though we can't identify all the genetic causes of diseases, thankfully there

are many researchers out there who are working hard to understand more about new techniques and working to identify new IRD genes.

Hopefully, what I have gotten across in the last couple of minutes I have been talking to you is that you understand genetic testing is an important part of clinical care for patients affected with IRD. I hope you have learned some additional information about the how and why in genetic testing and not only individual care but also the broader IRD community. I'm going to end there, thank you very much.

Jason Menzo, Chief Operating Officer

Thank you so much, Kari, for providing that awesome summary. I think everyone can appreciate with the increasing numbers of therapies being developed, how an important an accurate genetic diagnosis is and how critical it is for any person affected with an inherited retinal disease. That is why we are so encouraged by the number of patients enrolling in the My Retina Tracker Registry and why it's such an important initiative for us to continue.

Enrolling in the Registry not only can give you access to genetic testing and to a counselor like Kari, but it helps us to match individuals within the Registry and help progress in our field. It is about 20 minutes until 2 o'clock on the East Coast so we've got about 20 minutes available for Q and A, which is terrific as we have a number of questions in chat. We are going to open up the call and take your questions and comments and now I am going to ask Chris to please provide instructions for asking questions.

Chris Adams, Vice President, Marketing & Communications:

Thank you, Jason. There are several methods for asking questions. First, you may access the Q and a and chat features located at the bottom of the control bar and type in your questions. Second, you can ask questions verbally, select the hand raising function at the bottom of the interface and we will provide you with instructions to them yourself. You can press *9 to raise your hand pressing *6 will mute and unmute your line. You can also some your questions at info@fightingblindness.org. Please note, if there are questions that we were not able to answer on today's call due to time constraints, we will follow-up with you directly over the next week or two.

Jason Menzo, Chief Operating Officer

While we are compiling questions, I would like to let you know that in addition to our speakers on today's call Ben, Peter, Chris, and Kari, we are pleased to have Dr. Todd Durham, our Vice President of Clinical & Outcomes Research, and Dr. Amy Laster, our Vice President of Science and Awards Programs, joining us to answer questions.

I'm going to direct our first question that has already been chatted in to Todd. There's a very specific question about update related to a clinical trial called LUMIUS. Todd, can you just address a quick update on that particular trial.

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

The information I have today on the LUMIUS clinical trial is from publicly available documents both from the corporate website of MeiraGTx and the clinicaltrials.gov listing. The status of this Phase 3 study of X-linked retinitis pigmentosa due to the RPGR gene is that they have not yet beginning begun enrolling. From the information provided in clinicaltrials.gov, it looks to be a study where they're evaluating efficacy and safety primarily over 52 weeks. It looks like they're planning to study 48 individuals ages 5 and older males and females. That's the information we have to date. The company did provide a fairly comprehensive slide deck on their Phase 1/2 results that led to the initiation and design of this Phase 3 study. They are evaluating efficacy with regard to perimetry. They don't specify which type at clinicaltrials.gov. We look forward to seeing that study progress.

Jason Menzo, Chief Operating Officer

Thank you so much, Todd. Next, there are number of questions that have been chatted in related to specific gene mutations. I think everyone can appreciate some gene mutations affect a wide number of people whereas others are more rare or classified as ultra-rare. Not all of these different gene mutations have a specific gene therapy in development but here at the Foundation we have developed a portfolio of potential assets that are what we called crosscutting approaches that are agnostic to the gene mutation. Ben, can you give a brief update on some of those strategies that can affect a variety of different gene mutations.

Dr. Ben Yerxa, Chief Executive Officer:

We at the Foundation are fully aware that we have 300 different gene variants identified causing disease and counting. A good portion of the time someone is a genetic test it doesn't always come back as something definitive. As a primary strategy at the Foundation, we have put a lot of effort and funds behind crosscutting therapies. What we mean by this is therapies that could work broadly for IRDs regardless of the genetic driver of the disease. This is clearly a very efficient way to fund research, it is challenging, but it is really important that we have these strategies, because we have an understanding that a lot of people don't have a definitive diagnosis but if they do have a definitive diagnosis with a genetic test, there may not be a gene therapy that is being developed for that. At least, not yet at this time. We put a lot of effort into neuro protection, like rod-derived cone viability factor being developed by Sparing Vision in Paris, antioxidative approaches of neuroprotection, from Nacuity, and also optogenetics, which operates independently of a gene defect as a way to restore vision and the eye later in the disease cycle where there is a loss of photo receptors. Whenever there is an opportunity to develop a therapy that operates independent of the genes causing the disease, we are going to take a shot at it.

Jason Menzo, Chief Operating Officer

Thank you so much, Ben. We have a variety of different questions coming in and it's really the same question, Kari, that I'm going to direct towards you. How does one get in touch with a genetic counselor, ophthalmologist, do they contact a genetic counselor directly, what's the modality that an individual can get in touch with a genetic counselor.

Kari Branham, Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling, University of Michigan's Kellogg Eye Center

Genetic counselors are everywhere throughout the United States. There are genetic counselors that are located worldwide. There are couple of different ways that you can go about doing that. You could locate one yourself. The National Society of Genetic Counselors has a listing available on their website and that is

www.nsgc.org. That's one possibility. You could ask your primary doctor or your ophthalmologist for a referral to a genetic counselor as well. I would say that most large academic medical centers and/or hospitals in larger cities are going to have genetic counselors at them. Another possible mechanism, there are companies available that can provide telemedicine, so Informed DNA is an example, with genetic counselors who can see everyone across the country. You do need a referral from a healthcare professional, from a primary care doctor or ophthalmologist, just to have that consultation with the genetic counselor but that makes it accessible for everyone, everywhere.

Jason Menzo, Chief Operating Officer

I'm going to direct the next question to you as well. There is a question about times where someone gets a genetic test but it comes back inconclusive. Maybe you can speak a little bit more to the percentage of the time that that happens and alternately what an individual should do.

Kari Branham, Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling, University of Michigan's Kellogg Eye Center

If we look across all our patients, with all the different diagnoses that we see, RP, Stargardt, Ushers, about 60% of the time the results will be positive, with about 40% being negative or inconclusive. I would say that we probably actually get a larger portion that are inconclusive as opposed to negative meaning that nothing was identified.

Next steps for that. If they are negative there is nothing you can really do about it. Sometimes, if they are inconclusive, testing other members of the family can be helpful in determining which exactly is the cause of disease in the family. You would expect if there are other affected members of the family, they should have the same genetic variance, and other unaffected members of the family should not have that variance. It can help determine what is disease causing and what is not disease causing. Sometimes, if there is a genetic variant that we are not exactly sure if it is a cause of disease or not, sometimes over time those genetic variants do get reclassified as well. That is the other possibility. Sometimes you have to do work with other family members and sometimes it's a matter of time and we just don't know enough about the variance at that time.

Jason Menzo, Chief Operating Officer

I will throw one more at you. This is specific to age related macular degeneration. We talk about genetic testing for inherited retinal diseases but I am curious about AMD.

Kari Branham, Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling, University of Michigan's Kellogg Eye Center

When we think about age related macular degeneration, as opposed to other sorts of inherited retinal diseases, the main difference will be what is the cause of the disease. When we think about age related macular degeneration - it is considered to be a complex disease. That means there will be multiple changes in multiple specific genes we call them susceptibility genes. This means it is not just one gene or mutation in one gene that will cause someone to have the disease it is kind of a risk calculation for multiple genetic variances in multiple genetic genes combined with environmental factors, among things like smoking, that's considered a risk factors; vitamins are considered a benefit. All of these environmental factors together with the genes are going to determine someone's risk for developing that condition. That is compared with the conditions like RP and Stargardt where mutations in one gene will cause that person to be affected. In most of the cases, if not all, if those are genetic change then you will be at risk for developing the disease.

There have been a couple of publications that have come out, including one from the American Academy of Ophthalmology, that says we don't quite know enough about what to do for AMD, so generally not recommending doing tests. There are genetic tests out there that can give some estimation of this risk, but generally, there have been several publications that have been showing it is not really recommended by the large groups at this time.

Jason Menzo, Chief Operating Officer

We have a couple of questions specific to our My Retina Tracker. One is from an individual that had chatted that they are on Medicare. Curious if Medicare covers the cost of genetic testing or My retina tracker Open Access program obviously

covers this cost. Todd, can speak a little bit about the program, how individuals might find a doctor and what that process looks like?

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

Kari may add more as she may be more familiar with the requirements about Medicare and so forth. The open access program is available through Blueprint Genetic's website. They have a page called My Retina Tracker program which is this open access program. You need to visit an ophthalmologist or eyecare professional - someone who can diagnose an inherited retinal condition. That is one of the stipulations for accessing this program. They go through the Blueprint's ordering process and there's website to do that and they can order this panel for you. If your ophthalmologist or eye care professional does not have access to a genetic counselor, you will be referred to a genetic counselor at Informed DNA where you will get a professional genetic counseling session. You will have all the benefits that Kari just described a little bit ago. Kari, is there anything to add about Medicare?

Kari Branham, Clinical Assistant Professor and Director of Ophthalmic Genetic Counseling, University of Michigan's Kellogg Eye Center

Medicare can be challenging in that we have trouble getting prior authorization for the testing. It will depend a lot on the plan - some people have straight Medicare and some people have Medicare Advantage plans. If you didn't do the testing through the Foundation's open access program, there can be financial assistance programs for patients who have Medicare or Medicaid that some people will be eligible for. So even if it is not covered, you have the options like the open access program or financial assistance programs that can sometimes help with that.

Jason Menzo, Chief Operating Officer

Thank you, Todd and Kari. Amy, I'm going to address the next question to you. Today Ben reviewed several translations awards. How does someone find out about the awards we are funding?

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

There are two ways in which you can learn about them. Typically, when we make new awards, there is an article that is put out on the Eye on the Cure news report. So on April 9, the projects that Ben mentioned earlier, were published on our website.

More formally, under our funded grants, we are right at the end of the year where we are making a lot of new awards. In August of every year, we will update that with all awards made from the previous year. If you want to know what is happening as they come out, the Eye on the Cure news articles are your first place and then you can look at the funded grants for our complete set of funded projects.

Jason Menzo, Chief Operating Officer

We have a number of questions that have been chatted in. I recognize we only have about four minutes left. I do want to take this opportunity to remind everybody that after these calls we go through every question that has been asked - those that are addressed here on the call and those that we do not address on the call. We do follow up with every individual. Please recognize that if you asked a question and we didn't answer it on this call, we will follow-up with you to make sure that we give you those answers.

There was one that came through Facebook that is not related to an inherited retinal disease. It was related to something called a retinal detachment. The question is does the Foundation play a role in retinal conditions that are not genetic or inherited. The answer is no. We focus on funding research in inherited retinal disease, however, that said, if you would like to reach out to us at info@fightingblindness.org, based on where you're located, we could certainly direct you to a retinal physician that will be best in their class to be able to help you.

If there are ever questions or opportunities for us to help connect you as individuals in the local community with a retinal specialist, optometrist or low-vision center, that's part of the service that we do offer. Whether it's an inherited condition or not, we are lucky to have some of the best ophthalmologists and optometrist in the world.

Keeping an eye on the clock, what we are going to do is use this as an opportunity to remind everyone of some of the resources that we have here at the Foundation. We have mentioned it several times today but just to mention it one more time, our website fightingblindness.org is a great resource. We list all of the awards that we are funding, we also list all of the advancements that are happening in clinical research trials, whether we fund them or not. It is a great place to get updates on everything that is happening in the research landscape. It is also a place where we have education and background information on the different disease states. It is certainly a great place to find out about events like today but also our fundraising events we talked earlier in the call. Our Facebook, LinkedIn, Instagram pages are all great resources for learning about the latest developments in the retinal degenerative disease space. If you have any specific questions about your diagnosis, disease, gene mutation or information about specific genes, you can find more information on our website under the Newly Diagnosed or Retinal Disease sections.

We can also help connect you to our local physician referral program, which is available on our Resources page on our website and you can always reach out to us by emailing us at info@fightingblindness.org.

As we celebrate our 50th anniversary, the message that we are really emphasizing is that together, we are winning. That captures the essence of our many constituents, including our national organization, the robust scientific community, our chapters, volunteers and donors coming together to help fight in our march toward meeting our mission.

We would like to thank everyone for participating in today's call. As a reminder, there will be a transcript and an audio recording of today's call on our website within the next week. If there is any other information that you need or questions that you have, please reach out to us by sending us an email at info@fightingblindness.org. Thank you so much and have a great rest of the day.