

**Foundation Fighting Blindness**  
***Insights Forum* Transcript**  
**January 31, 2020**

**Chris Adams, Vice President, Marketing & Communications:**

Good afternoon. Welcome to the Foundation Fighting Blindness Insights Forum Call. I am Chris Adams, Vice President, Marketing & Communications at the Foundation. We appreciate everyone joining us. I would like to review some of the logistical details we have for the call. Currently, all lines are in listen-only mode. Today's conference is being recorded and is available in closed captioning. To activate the closed captioning, select the option at the bottom of the Zoom interface. Please note that today's event does not have any slides. It is purely an audio presentation.

For those of you using screen readers, please be aware that the controls are at the bottom of the Zoom interface. This bar may collapse when it's not in use. If you prefer to prevent the controls from auto-hiding, please go to the settings within the Zoom platform, select accessibility and then select "always show controls".

To ask questions during the event, you can use one of three methods. You can activate the Q&A feature on the Zoom control bar to ask questions. You can ask them verbally. Click the raise your hand button, and we'll provide instructions on unmuting your line. Third, if you joined in by telephone only and are not on the Zoom application, please submit your questions via email to [info@fightingblindness.org](mailto:info@fightingblindness.org). At this time, I would like to turn the conference 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. I'm the chief operating officer here at the Foundation Fighting Blindness. I'd like to welcome you to our quarterly Insights Forum call. The purpose of these quarterly calls is to highlight all the latest developments here at the Foundation Fighting Blindness and provide updates on our progress towards our mission: which is to drive and accelerate the search for preventions, treatments and cures for inherited retinal diseases.

On today's call, we're going to start with an update on our community outreach and public awareness activities, followed by a summary of our recent financial

performance. Then we'll have a very special presentation led by our CEO, Dr. Ben Yerxa, who will discuss a comprehensive overview of the many current clinical stage programs in the inherited retinal disease landscape. With more than 30 clinical trials ongoing in our space, it's a great opportunity to update you on what's in the pipeline for new treatments.

After the presentation, we'll have a question-and-answer period. Chris will repeat the instructions on how to ask your questions. As mentioned, this call is being closed captioned. A replay and transcript of this call will be available on our website in the weeks ahead. If you have any feedback related to accessibility or other suggestions for the call or the Foundation in general, please reach out to us at the email address: [info@FightingBlindness.org](mailto:info@FightingBlindness.org).

Now I would like to begin our call by sharing progress we've made in the area of public awareness and community engagement. A key part of our mission is to help make connections between the various stakeholders in our community.

In order to help make those connections, we have been working very hard to raise awareness of the Foundation in our community through a Public Service Announcement (PSA) campaign.

The direction of this campaign is all about individual empowerment and speaks to the idea that no matter what a person's diagnosis may be, it's their drive that can propel them to accomplish great things and follow their passion. With everyone in our community working together, we are stronger, and we can accomplish great things.

The national PSA campaign launched officially in late October. Since then, the TV ad has run – wait for this – more than 25,000 times in nearly 170 markets on over 280 television stations across the country. The top TV markets where the PSA has run include some of the biggest markets in the nation including New York, Los Angeles, Dallas-Ft. Worth, Boston, San Francisco and Chicago. This week, we also had some great news that our television spot was picked up by ION Network as well. Our radio spot has run 2,500 times on 65 radio stations nationwide.

Remember that PSA ads are free to the sponsor, so all these television and radio spots have come at no cost to the Foundation. The combined benefit of this coverage is estimated to have received over 480 million impressions; which translates to nearly \$11 million in free media, which is an incredible thing to consider.

Another major initiative we've recently announced is the rebranding and relaunch of our signature fundraising event, the VisionWalk. More than 35 walks are planned for

this year alone. They've raised over \$55 million to help advance our mission.

This year marks the 15th anniversary of VisionWalk. We felt there was no better way to celebrate than to inject new energy with a fresh, bold new look. Our new logo utilizes a color palette of deep blue, which represents trust, unity and importance, with the contrast of turquoise, which signifies patience, protection, tranquility and healing. An accent of gold will be part of the new branding which represents the guiding light and signifies a beacon of hope and strength for those who are affected with a blinding retinal disease. Importantly, this new branding is an extension of the Foundation Fighting Blindness branding and logo that we launched this time last year.

In addition to the relaunch of VisionWalk, we have been working to make other fundraising easy for families and their supporters. We rolled out a new DIY fundraising platform that creates the ability to host a pancake breakfast to a championship golf tournament and literally everything in between. We've created templates powered by our new fundraising system, Classy. You can find out more information about this DIY campaign on our website, [www.fightingblindness.org](http://www.fightingblindness.org), under the "Get Involved" tab on the homepage.

Now I would like to shift gears and take a moment to share an inspiring story about a special family that's been successful in organizing their own DIY fundraisers for many years. Many of you know the Bergstein family. Like many of the families in our community the Bergstein's have multiple generations diagnosed with retinitis pigmentosa (RP).

For almost 20 years, Jordan and his wife, Paula, have organized a golf tournament, actually going back to the year 2000. This is a fundraiser for the Foundation. After many years of success, they decided to pass the torch on to their son, Jason, and their daughter, Bari. When Jason was affected by the disease, they chose to shift gears, and organize a family-friendly bowling event in lieu of the golf tournament. Since 2015, this annual bowling fundraiser has raised nearly \$400,000 for our mission.

I wanted to use this opportunity on the call today to thank the Bergstein family and hopefully inspire all of you on the call to consider your own DIY fundraiser event as well. The new platform is live on our website at [www.FightingBlindness.org](http://www.FightingBlindness.org) under the "Get Involved" section.

Another initiative we kicked off in 2019 was the creation of our Strategic Council, a group of young professionals from across the country with an affinity to our cause and represents the next generation of Foundation volunteer leaders. This diverse group includes lawyers, entrepreneurs, marketing and finance executives, and Ivy League

professors. They're a rock star group of folks.

The Council was developed to collaborate and problem solve some of the challenges and opportunities we are facing today – including how to engage and grow our participation among a younger population. We're very grateful for their volunteer service and commitment. A lot of work is underway with their leadership. If you or someone you know would like to learn more about serving on the Council, send us an email to [info@fightingblindness.org](mailto:info@fightingblindness.org).

We focus a lot of time working with families with inherited retinal diseases and the healthcare professionals working with them. We now have an amazing Outreach team that continues to make strong connections with eye care professionals and groups serving the low vision community. Last year, this team participated in the annual meeting of the VisionServe Alliance, comprised of organizations serving individuals with low vision. Thanks to the connections made at that meeting, our team has been invited to deliver a presentation to more than 120 CEOs and executives of related organizations within the low vision community to highlight the Foundation and our resources for people living with inherited retinal disease.

We continue to increase awareness and participation in the Foundation's My Retina Tracker Registry. As many of you know, this is an online patient database, that anyone with inherited retinal disease can join to help accelerate research in clinical trials. A quick update on the Registry - it currently contains more than 14,500 members of which over 6,500 have been genetically tested.

An essential companion to our Registry is the My Retina Tracker Genetic Testing Program, which is gaining significant interest from eye care professionals as well. This is a new nationwide program launched this past fall. It allows any clinician who has diagnosed an individual with an inherited retinal disease to order a comprehensive, high-quality genetic test and genetic counseling for no cost to the patient. Any person with an inherited retinal disease may be eligible. We've had multiple educational seminars to educate doctors on how to enroll their patients.

Finally, I would like to focus on the events coming up. This spring, we will host 20 VisionWalks, 15 dinner galas, wine events, and other local fundraisers all taking place across the country. We will be hosting our Vision Seminars in Austin, Fort Wayne, the Bay Area and Atlanta. They provide updates for attendees on living and thriving with inherited retinal diseases.

We're hosting several national meetings, including the Investing in Cures Summit on March 13 & 14 in Raleigh, North Carolina. This event is focused on bringing together a

cross section of industry partners, investors, translational research experts, clinical trial investigators, and the companies poised to take therapies across the finish line.

We're also hosting the 8th annual Retinal Cell and Gene Therapy Innovation Summit in Baltimore, Maryland, on Friday, May 2, in conjunction with the Casey Eye Institute at Oregon Health & Science University. The Summit takes place just prior to the 2020 annual meeting of the Association for Research in Vision and Ophthalmology (ARVO). At this Summit, representatives from biotech and pharma industries come together with members of the physician and research communities to discuss rapidly emerging ocular therapies and strategize how to move the most advanced retinal disease therapy options forward.

And finally, our flagship event is coming up this summer, VISIONS 2020. We are very excited about our upcoming VISIONS Conference, which we host every other year, bringing together our global inherited retinal disease community. This year's conference will be held from June 18-20 at the Hyatt Regency Minneapolis. Our team has put together a comprehensive program of scientific presentations, clinical updates, patient-focused sessions and recognition of community member achievements. Online registration for the Conference is now open. For more information, go to [www.FightingBlindness.org/VISIONS2020](http://www.FightingBlindness.org/VISIONS2020). We hope you will all join us!

I would like to provide a brief summary on our current financial position. The Foundation operates on a fiscal year from July through June. So our 2020 fiscal year will be coming to an end on June 30, 2020.

As of December 31, 2019, our actual fiscal year-to-date revenue was approximately \$11.8 million against expenses of \$7.0 million. We remain on track to achieve our budget for fiscal year 2020, which includes targeted revenue of \$25.6 million against expenses of \$15.3 million, with greater than 70% of those expenses going directly to mission related efforts, including \$8.8 million to support brand new research projects.

Our team at the Foundation is working closely with our community stakeholders to generate funding for this research. We appreciate all your time, talent and resources to help us. We really are stronger together.

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

**Dr. Ben Yerxa, Chief Executive Officer:**

Thank you, Jason. Good afternoon and thank you for joining us on our quarterly update call for the inherited retinal disease community. Our urgent mission is to drive the research that will provide prevention, treatments and cures for people affected by retinitis pigmentosa, age related macular degeneration, Usher syndrome and the entire spectrum of retinal degenerative diseases.

At the beginning of a new year and a new decade, it's an ideal time to reflect on the progress that has been made to date and the exciting opportunities that are advancing rapidly in the retinal degeneration field.

Twenty years after the Foundation was established, the genetic basis of inherited retinal disease started to be unraveled with the discovery of rhodopsin's role in causing RP and then later many genes, now up to over 272 and counting. The prevalence of IRD's is about 200,000 patients in the U.S. and worldwide, it's over 4 million. Each individual disease is certainly an orphan indication but as you roll it all up it gets to be a fairly large population of people.

On the regulatory front, we have also made progress. The Foundation and other supporters of the ophthalmology division of the FDA wrote letters to the Director of the Center for Drug Evaluation and Research at the FDA, Janet Woodcock, to advocate for the ophthalmology division to have a direct reporting relationship to the head of the Office of New Drugs, rather than being combined with other divisions such as transplants and anti-infectives. That reorganization was completed in early 2018 and now Dr. Wiley Chambers, who's the head of the Ophthalmology division, now reports directly to the head of New Drugs at the FDA.

I'd like to provide you with a 2020 "state of IRD programs" that are in development. I'll go alphabetically by genetic-related disease. It's going to go fast, but I want to be as comprehensive as possible. As a reminder, we will post a transcript and audio replay of this call so that you can find the specific details on the update that we are providing today.

Achromatopsia - CNGA3 – there are a couple of programs here; AGTC recently announced some interim results last week; it looks like they need to go a little bit longer in duration to see the results fully play out but it looks somewhat favorable so far. MeiraGTx is partnered with Janssen, a Johnson & Johnson company, and is conducting a small Phase 1/2 trial. That trial was initiated in the 3rd quarter of 2019 and we are awaiting results.

The CNGB 3 type of achromatopsia has a similar story with AGTC reporting out some initial results and MeiraGTx in a small trial of about 23 patients with dosing complete. We are awaiting the data read out.

In Dry AMD, we'll start with some recent movement in the anti-complement field. There have been recent positive results from Iveric Bio with their anti-C5 Zimura drug and Apellis with their Phase 2 FILLY trial results that came out. Apellis has 2 large Phase 3 trials underway that that won't read out for another year or so, and Iveric plans to announce their Phase 3 plans later this year.

Dry AMD is also heating up in the gene therapy area where Gyroscope launched the first ever gene therapy trial for dry AMD in early 2019 and then there's some other companies working in this space, including Gemini working on Factor H and Factor I and some start up activity coming out of University of Utah with Dr. Greg Hageman.

In terms of cell therapies for dry AMD, there's also some significant movement here with work done by Astellas working on pluripotent stem cells, CellCure out of Israel and Regenerative Patch making the cover of Science Translational Medicine with their very interesting RPE cell-based technology.

Also in dry AMD, a company called Allegro, that has an anti-integrin approach, announced results in July 2019 showing us that the subjects in this trial actually gained some vision. So those are pretty interesting results.

Moving on to BEST1 disease, Iveric Bio recently licensed the gene therapy technology from University of Pennsylvania and they plan to be in the clinic in the first half of 2021. It's great to see that technology being picked up by industry.

Choroideremia - REP1 – there are two programs to note here. Biogen, by way of their acquisition of Nightstar, is in Phase 3 with their STAR trial in about 140 patients. We are awaiting the results for that trial which was initiated in early 2018. In addition, Spark Therapeutics has an early Phase 1/2 trial that has yet to read out.

Enhanced S-Cone Syndrome – this is the Nr2e3 target. A new company on the scene called Ocugen, recently public, has licensed and is developing technology out of Dr. Neena Haider's lab at Harvard. We're very interested to see how that program progresses.

Now let's go through the LCA's or Leber Congenital Amaurosis.

Starting with LCA-1, which we also call GUCY2D. This is under development by Sanofi licensed from University of Florida and they've started a Phase 1/2 trial that's actively

enrolling under a US IND.

LCA2 or RPE65 – there is the LUXTURNA approval that most everyone knows about, but it's also worth noting that MeiraGTx in the UK has a program with a Phase 1/2 complete and we are awaiting an update from the company on that trial.

LCA4 (AIPL1) - this is a very small population, but this is one where MeiraGTx has actually developed this and is made it available via a “specialist license”. This is a way in the UK to make medicines available on a special case basis, so if anyone knows anyone with the AIPL1 gene, you have to catch it early but there is something that's potentially available to treat that condition.

LCA5 (the lebercilin gene) – there is some recent activity here with a new company called Limelight Bio that licensed this program from Dr. Jean Bennett's lab at Penn, and it's in pre-IND development.

LCA6 (this is RPGRIP1) - some recent work coming out of Harvard including some work by the Dr. Luk Vandenberghe. This was licensed by PTC Therapeutics via a nonprofit therapeutics company, Odylia, that is working on gene therapies in the eye.

LCA8 (CRB1) - some interesting activity here. Dr. Jan Wijnholds has been working on this program for a long time, and there's some new recent publications coming out of Dr. Bennett's lab and also Dr. Jeremy Kay had some work shown at ARVO and a recent nice review article in an ophthalmology publication. This space is starting to heat up. We will be able to give an update hopefully in the next year or so with some more developments.

LCA10 (CEP290) - the company ProQR, based in the Netherlands, has started a Phase 3 trial after reporting out positive Phase 2 data; there's a gene editing approach via Editas and their partner, Allergan, in Phase 2 which is recruiting – this is the first ever gene editing trial in which the therapy is injected into the body. Then Iverica Bio has a preclinical program with some technology coming out of Dr. Hemant Khanna's lab at University of Massachusetts. And last but not least, Oxford BioMedica also has a program that is in pre-clinical development.

LCA13 (RDH12) – the Foundation Fighting Blindness worked with a patient group to conduct an RDH12 workshop in 2019. It was a great way to showcase the power of advocacy with the patient groups pulling together a really nice review of the literature that was presented to all the attendees ahead of time. Work was showcased from Dr. Debra Thompson's lab and there was participation from Dr. Bennett's lab and industry including MeiraGTx and Limelight Bio both attending. Importantly the FDA's head of



ophthalmology, Dr. Chambers, attended the entire day with the group, providing a lot of great regulatory input. We also talked about novel endpoints and Natural History studies. It's a really good model for us, and we're happy to do 1 or 2 of these a year to the extent we can work with an organized group.

LCA (NPHP5/IQCB1) - there has been some activity here. There's been some work by Dr. Wolfgang Baehr at Utah that's been published. Then also more recently with Dr. Gus Aguirre and Dr. William Beltran working on a gene therapy for this particular variant and we look forward to hearing more about that.

Leber Hereditary Optic Neuropathy - a little bit about LHON. This is not typically work that we have funded, but we wanted to take note of work done by GenSite, where they have recently come out with some promising Phase 3 results showing some bilateral improvements in vision, so it's worth noting some of the progress there.

Moving on to Retinitis Pigmentosa.

RP1 - there's some work done by Oxford BioMedica with a lentiviral gene therapy that's pre-clinical. Also, Dr. Peter Campochiaro has been doing some pilot work with oral N-acetyl cysteine with a 30-patient open label trial that was recently published. We will look forward to seeing what the long-term results might be from that approach. Interim results were recently published.

RP2 - this is some work done at the National Eye Institute in collaboration with University of Massachusetts Medical School with some gene replacement therapy in animals being published recently. We await see if someone picks that program up.

RP - PDE6beta - this is a program that came out of France. It's been licensed by a company called Horama that focuses only on IRDs. They're currently enrolling a Phase 1/2 trial, and it's all being done in France.

RP (RLBP1) - this is a gene therapy program that was homegrown within the walls of Novartis, so it's great to see big pharma in the mix here. This trial is up and running in Sweden and we await to hear some results from that one in the near future.

For RP cell therapy there are 2 ongoing trials with retinal progenitor cells; jCyte has an ongoing intravitreal dosing Phase 2b trial that won't be reported out until ARVO in early May. Reneuron is doing subretinal injection of similar kinds of cells with an open label dose escalation trial of 3 different dose levels. They have released some interim results with some noted improvements in vision but also some adverse events that need to be carefully watched.

For RP photoreceptor regeneration, there are several approaches here. There's work being done by Dr. Tom Reh and a new company called Nayan Therapeutics taking on the approach of Nr2e3 modulation with small molecules, which is in preclinical development. Dr. David Gamm, at University of Wisconsin, has a spinout company called Opsis, that's working on combination products of RPE and photo receptor cells and we are interested in those updates as they come. Dr. Mike Young at Harvard is also continuing to work in this area and we look forward to hearing more about that.

RP Rho – there has been some recent activity here. ProQR has licensed a program from Ionis and this allowed them to go straight into the clinic in late 2019 in a Phase 1/2 trial. This is essentially an antisense oligo approach and we await the results from that trial.

RP - RdCVF - this is rod derived cone viability factor. There is a company, Sparing Vision, that was formed from the lab of Drs. José Sahel and Thierry Lévillard in Paris, France. The Foundation has invested in this gene therapy company and they are preparing to get into the clinic in 2021. This is essentially a neuroprotection approach that is gene agnostic.

RP Usher Syndrome - another company that the Foundation has funded is Nacuity Pharmaceuticals, which has an oral broad antioxidant neuroprotective approach. They are now enrolling in a Phase 1/2 trial in RP associated with Usher Syndrome in Australia, so we await results from that trial in the next one to two years.

RP Anti integrin - there has been some work done by the company, Allegro. Though they are initially focusing on diabetic macular edema, they have announced that they have some Phase 1/2 proof of concept work in retinitis pigmentosa. We'll wait to hear some results on that particular approach hopefully in the next year or so.

Moving on to Optogenetics - this can really apply to retinitis pigmentosa or AMD and there are several companies that are in the clinic. Allergan, through their acquisition of Retrosense, haven't reported any new results since the earlier results were presented prior to the acquisition, but we understand that this program is continuing in development. Gensight also has an optogenetics program that requires wearing goggles to amplify the signal. We await the results from that trial as well. There is next generation work in optogenetics from two companies currently. Acucela is working on a way to transduced bipolar cells and Vedere Bio, which the Foundation has recently funded, has next generation work coming out Dr. John Flannery and Dr. Udi Isacoff's labs at UC Berkeley.

In Stargardt, there is a lot of clinical work going on.

There are several approaches with gene therapies for the ABCA4 gene. Sanofi has the StarGen lentiviral-based approach that has been in Phase 1/2 for while. We are waiting for this program to get picked up by some other industry partners as Sanofi is getting out of ophthalmology altogether. Spark Therapeutics also has a program in the discovery phase that has been mentioned briefly in public materials.

For Stargardt, there are several visual cycle modulators in development. Acucela has emixustat in a Phase 3 trial with 160 patients, so we await results from that one. And Alkeus has an interesting deuterated vitamin A approach that's in the clinic and we await readout from that trial.

There are also next generation visual cycle modulators - this is the RBP4 target. There's Belite Bio, which has completed Phase 1 and entering Phase 2. And Stargazer is entering the field now but hasn't released any R&D details.

For Stargardt Cell Therapy - Astellas has a program that is essentially the same as what they are doing in AMD but they have enrolled some patients with Stargardt disease in their Phase 1/2 open label, dose escalation trial with this pluripotent stem cell approach. And also of note, in terms of a complement approach, Iveric Bio's drug that is being used in dry AMD related geographic atrophy is also being applied in Stargardt disease. They are conducting a fairly large Phase 2b trial with 95 subjects that is expected to read out in the second half of this year.

Usher Syndrome 2A – specifically Exon 13 – there's an important program by ProQR that's in a Phase 1/2 trial that's going to read out in the next several months, so we await the results from that program that the Foundation has co-funded.

Usher 1b -Myo7A Dual AAV Vector – there are a couple of approaches including Dr. Shannon Boye's lab at University of Florida and Dr. Alberto Auricchio's lab in Italy working on ways to deal with this very large gene. There is also the UshStat Phase 1/2 trial from Sanofi that is also awaiting an out-licensing partner to move it ahead.

XLRP – RPGR - AGTC recently announced 6-month data from a Phase 2 trial showing some improvements in vision that's quite encouraging. Others are working in this field as well, including MeiraGTx partnered with Janssen. They've got a Phase 1/2 trial ongoing. And Biogen through their Nightstar acquisition has an adaptive phase 1/2/3 trial ongoing, which started in March of 2017.

XLRS - RS1 – The program AGTC partnered with Biogen is winding down after some disappointing results in 2019 with intravitreal dosing. There's a program at the National Eye Institute that's enrolling up to 24 patients in an open label Phase 1 trial.

I know this has been a rapid-fire review, but we wanted to give you a flavor for how much science in business we're tracking for you at the Foundation. We plan to provide a periodic comprehensive written overview of this progress that will allow us to update you on a more regular basis.

We are very excited about the breadth and potential of research happening across academia and industry. A key part of the Foundation's opportunity to advance treatments for IRDs is our Retinal Degeneration Fund, which invests in companies with projects that can be in clinical testing in 18 to 24 months. The RD Fund currently has more than \$70 million in funding and has deployed that in six companies for a total of \$42 million in currently committed capital and reserves. It is truly inspiring to see the innovation these companies are developing. You can find more information about the fund online at [www.RDFund.org](http://www.RDFund.org).

In summary, we at the Foundation want to thank everyone in the IRD community for all that you are doing to help prevent and treat these blinding diseases.

I'd now like to turn the call back over to Jason Menzo, our Chief Operating Officer, so we can take your questions.

**Jason Menzo, Chief Operating Officer:**

Thank you, Ben. That was amazing. That was a lot to cover. There will be a test. We should have said that at the beginning of the call. I know some of you listening today, were wondering whether there were slides. There are no slides for this presentation. This presentation and all of our quarterly Insights Forum calls are audio only. That said, everything that we discussed today will be available on the website both through a recording and through a fully accessible transcript. So even though that was a lot to cover, we'll make sure that everyone who is interested has access to the material that was covered today to go back and rereview.

We will now open the call and take your questions and comments. I will turn the call back to Chris to provide the instructions for asking questions.

**Chris Adams, Vice President, Marketing & Communications:**

Thanks, Jason. As a reminder, there are three methods that everyone can ask questions. You may access the Q&A feature on the bottom of the Zoom control bar. I see those

coming in now. Secondly, you can ask questions verbally. Select the hand raising function at the bottom of the Zoom interface, and we'll provide you with instructions to unmute yourself. You can submit your questions to us at [info@fightingblindness.org](mailto:info@fightingblindness.org). If we don't get to your questions due to time constraints, we'll follow up with you.

**Jason Menzo, Chief Operating Officer:**

While we are compiling questions, I'd like to take a moment to highlight some of the resources that the Foundation provides.

Our website, [FightingBlindness.org](http://FightingBlindness.org), our Facebook page, Twitter, LinkedIn and Instagram accounts are all great resources for learning about the latest developments in the retinal degenerative disease space. If you have specific questions about your diagnosis, disease or genetic information, you can always find more information on our website under the Newly Diagnosed or Retinal Diseases sections. We can also help you connect you through our physician referral program, which is available on our website under the Resources section. You can also reach out to us by just sending an email to [info@fightingblindness.org](mailto:info@fightingblindness.org).

I'm very excited to announce that for our Q&A session, we're joined by Dr. Brian Mansfield, the Foundation's executive vice president of Research and Interim Chief Scientific Officer. He'll be joining us for the Q&A session.

The first question I'm going to direct to Brian. The question is related to the natural history study for the EYS gene. It was said to have started last year. At last check, we were curious what the status is regarding recruitment. Brian, I'm going to turn it over to you.

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

Thank you very much. Good afternoon to everyone who is listening to this webinar.

Let me first start by giving a brief background on the EYS Natural History study, just in case people are not familiar with it. This is for people who have retinitis pigmentosa. The study is called Rate of Progression in EYS Related Retinal Degeneration. If you take the first letter of all of those, it comes up with the acronym Pro-EYS. If you want to intervene in a disease, you first have to know how the disease progresses to be able to demonstrate that, in fact, you've been able to change the rate of progression or, in fact,

stop it.

This is an international, four-year study which will include estimating the rate of disease progression and evaluating the usefulness of outcome measures for future clinical trials for emerging therapies. Our goal is to accelerate EYS therapy development for all academic and commercial researchers. We make data from the natural history study accessible to researchers who can use it to design clinical trials.

The study is a significant undertaking. We are grateful to the investigators, reading centers, laboratories, genetic experts, and the study participants who make it possible.

The study design for Pro-EYS includes three cohorts. It will include 70 participants with visual acuity of 20/80 or better and a visual field diameter of 10 degrees or more.

It will include approximately 20 participants with visual acuity between 20/100 and 20/400 or with visual acuity of 20/80 or better and visual field diameter of less than 10 degrees.

It will include approximately 10 participants with visual acuity of 20/500 or worse.

I realize those are difficult to hear and assimilate on the phone, but you can find them on the [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov) page.

After an initial baseline visit, participants will make four annual visits for exams and vision testing. Investigators will be evaluating several aspects of vision and retinal health including: visual fields, visual acuity, retinal sensitivity, and the size of the patient's ellipsoid zone, the area of healthiest photoreceptors.

The following collaborators will be supporting this multi-year study:

- Jaeb Center for Health Research
- Duke Reading Center
- Casey Reading Center
- Blueprint Genetics
- InformedDNA
- Emmes
- Kari Branham, MS CGC, University of Michigan
- Stephen Daiger, PhD, University of Texas
- Robert Hufnagel, PhD, NIH/NEI

There were delays compounded by the holiday season, but now our first sites are on track to be certified in the use of this new imaging procedure within the next two to three weeks. As soon as that certification is received, then patients will be able to be enrolled.

Additional sites are close to completing their certification process. Over the next month or so, as they complete that, they will start enrolling patients. If you're interested in participating in the study, the best point of contact is the coordinating center, at the Jaeb Center for Health Research. You can reach them at FFB@JAEB.org. You can call them at 813-975-8690.

I know there had been a question come up specifically about the Duke Reading center. They now have an enrolling coordinator. If you contact them, they will be able to give you all the information you need.

**Jason Menzo, Chief Operating Officer:**

Thank you, Brian. The level of focus on the call always rises with your accent. Thank you very much for that very comprehensive answer.

We have a number of questions that have been chatted in and emailed in. I know there are a couple on the phone line as well. We're going to try to move rapidly through many of these questions. I'm going to take liberty to try to combine the spirit behind several questions into one, so we can try to cover as much ground as we can. There are several questions related to Stargardt specifically.

The questions are related to the investments we have in Stargardt's disease. There's a question if we looked at it from a natural history study.

**Dr. Ben Yerxa, Chief Executive Officer:**

In terms of actual RD Fund investments, we haven't done that yet, although, we're very close to making an investment. So you have to stay tuned. It's still confidential right now. We're always looking for good opportunities in this space. Alkeus has been raising money. We would be interested in taking a look.

In terms of natural history studies, we completed the largest natural history study ever in Stargardt's. It was a multimillion-dollar study that's been published in journal articles.

If anyone wants an update on that, you can send in an email.

Brian, you can touch on different ways we've invested in Stargardt's to complete that picture.

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

I was looking at clinical trials focused on Stargardt's disease. You can find this on the [www.clinicaltrials.gov](http://www.clinicaltrials.gov) website. They range from cycle modulators to other techniques. It is a very active area in the clinic at the moment.

In addition to that clinical trial work, the Foundation has made substantial investments in clinical research programs for Stargardt's. We're supporting a significant amount of gene therapy work, specifically for the ABCA4 gene. It's too large to fit into the standard vector that's used in retinal gene therapy.

Dr. Shannon Boye and her team at University of Florida have come up with an innovative way of dividing the gene into two bits and delivering those two bits, each of which fit into a virus, into a cell, then putting the gene together again from the two parts delivered to it. That is going well. That technology has been licensed, and it's been announced they're going to be testing it in large animal models, which means that they're moving rapidly in translating research into clinical trials.

We have a researcher at Cleveland University, Dr. Liu, who is working on a way of delivery without a virus, using nano particles. We have funded a number of researchers who are looking at the second genetic defect in Stargardt's disease. We can often only find one mutation in the patient when we diagnose. To be a definitive diagnosis, you need two mutations in the patient. Dr. Kramer is being funded to develop ways of finding the second mutations which tend to be hidden in parts of the gene that are often not looked at.

I hope that gives a flavor of this type of work we're investing in both clinically and preclinically.

**Jason Menzo, Chief Operating Officer:**

Excellent. Thank you, Brian. We have just about 10 minutes in the time left together. I want to ask another research related question. There are several questions related to



Usher Syndrome. And two that I want to highlight, Brian. I want to ask you to speak to it from a 30,000 foot level and your knowledge of things happening with regards to the various subtypes of Usher questions, specifically what is being done in Usher's 3A. Also, a second question about what's happening in Usher's 2A. I'm sure there are others wondering about the various subtypes. Again, can you speak at a high level. You could probably go over an hour discussing it, but maybe things that will be applicable to folks affected with Usher Syndrome.

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

The Usher portfolio is large. You heard in Ben's presentation, talking about ProQR that have announced a therapy for a particular mutation, Exon 13 This is probably the most common cause of Usher, for people with an Usher 2A gene. Excitingly, that just received a rare pediatric designation from the FDA, which not only helps the company move this process through faster but has a benefit to the company if they're successful.

They're also developing a similar type of technology for a different group of mutations in the Usher 2A gene. That's the clinical work at the moment, but it's coming on rapidly and benefitting from what you already have.

In other Usher diseases, we have a lot going on in Usher 1B, including a lot of work with gene therapy using split vectors. As I explained before, we have specific mutations being addressed by read through drug where the mutation basically gets covered up by a small molecule, so it's not seen by the cell. Usher disease does not manifest in small animals, like mice, when we try to knock the disease out. So we're developing animal models - not only large animal models but small squirrel models. We're supporting clinical imaging to support the disease.

In Usher 1C, we have work going on. Dr. Jennifer Lentz is working at LSU to get that in.

In terms of more broad stroke therapies, it may not be specific to a unique gene but more to a type of disease such as RP, we have a number of programs. You already heard Ben talk about the work with Dr. Tom Reh where they're working chemical triggers that cause cells to die in the first place. He's developing a way to modulate those factors so the cell does not make that decision to die.

And then you've recently heard about work from Nacuity. They finished the Phase 1 trial in RP patients and showed that there is retaining of vision for six months. This is an antioxidant strategy. We have a couple of those approaches, also, in our portfolio.

As you said, there's a tremendous amount going on, but I hope that will give people a flavor of the sorts of things we're doing.

**Jason Menzo, Chief Operating Officer:**

Excellent. Thank you, Brian. I'm going to answer one more question sent in. Then we'll go to the phones in just a moment. There was a question regarding the legislation that we've talked about in previous Insight Forum calls. I'm sure everyone knows there's quite a bit happening in Washington these days. The political landscape is as challenging as ever to navigate. That said, there's a lot of activity at the Foundation under the leadership of one of our Board members, Karen Petrou, to have a lot of effort working through the House of Representatives with regards to this legislation. I'm also happy to report we have two lobbying firms working on this legislation. Not only Akin Gump, who has worked with us in the past, but we have another working in a pro bono fashion. We're building momentum at the committee level to take action with this bill. So, hopefully, in the next month or two, we'll have more to provide.

With that, I know we have one or two folks on the line. Let me ask you to answer those or bring up those who are on the phone.

**Chris Adams, Vice President, Marketing & Communications:**

Kevin Waller, you should be available. Kevin, are you there?

**Kevin Waller:**

I'm here. I have CRVO, diagnosed in 2005. Wondering if there's any research studies for that or new medications?

**Dr. Ben Yerxa, Chief Executive Officer:**

Kevin, this is Ben. You said CRVO? I'm not sure I have that at my fingertips, but, typically, that would be anti-VEGF. If you want to send us an email, we'll try to get you more information.

**Jason Menzo, Chief Operating Officer:**

Chris, is there anyone else on the line?

**Chris Adams, Vice President, Marketing & Communications:**

We are looking right now. We have a question that's in the Q&A box that we could take.

**Jason Menzo, Chief Operating Officer:**

I would say the majority of questions, which is totally understandable, are about specific genes or specific clinical areas. I will pick one. In general, I want to make sure everyone knows that everything that was typed in or asked, we do compile these. Every call, we spend a considerable amount of time going through each question, following up with each individual. So rest assured if we don't cover your question here on the call, we'll follow up with you directly.

I did want to pass one question on to Brian with regards to BBS1. Do you have anything you can share with regards to preclinical research that's happening in that arena?

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

There's nothing in the clinic yet for BBS type 1. There are a number of preclinical studies going on. A lot are in Ohio by Dr. Stone and Dr. Tucker. It would be a little difficult, I think, to go through each of the approaches at the moment on the phone.

**Jason Menzo, Chief Operating Officer:**

We will follow up on the email. One more, I'm going to direct to you, Brian. The second I will answer. The first was related to My Retina Tracker Genetic Testing study in terms of if an individual is interested in participating and having their physician request a genetic test, what that looks like, and how that happens.

The second is for those interested in joining the Strategic Council, how to go about that. We'll tackle that one, but, Brian, why don't you address the genetic tracking program.

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

If you would like to take advantage of the My Retina Tracker program, which is the open access genetic testing, any person who has a clinician who has diagnosed them with an inherited retinal disease can go to their clinician. The clinician is the key point of contact for you. Ask them to order the test for you. To do so, you can go to our website, [www.fightingblindness.org](http://www.fightingblindness.org). Under Resources, you will find a tab about genetic testing. There you will find information that you can either direct your clinician to look at or you can hand to your clinician. It tells the clinician how to go about establishing an account at the genetic testing company who will be carrying out the testing. There's only one company that does the testing. The company will only work with a clinician. They will not take calls from patients. The clinician needs to establish an online account with them. It takes them just a couple of minutes. He then orders the test for you. He takes the blood or saliva sample from you. He sends it in. You wait for the results. They will also be explained to you by a genetic counselor. There will be complicated results that come back. But they're skilled at putting it in lay terms in helping you understand what it means and how it affects your disease and the potential clinical trials you may be eligible for.

**Jason Menzo, Chief Operating Officer:**

Thank you, Brian. Not to have left the Strategic Council question as a cliff hanger, but, very simply, if someone is interested in learning about our Strategic Council or our programs or other initiatives, we have a general mailbox we go through daily and direct questions and opportunities to the right personnel on staff. If you're interested in the Strategic Council or learning more, send an email to [info@fightingblindness.org](mailto:info@fightingblindness.org).

It's just after 2:00 pm here in the east. I do want to remind everyone, especially if you joined the call midway through and you want a recap of the call, this call in its entirety will be available on our website both in an audio recording as well as a fully accessible transcript in the next couple of weeks. We will push out a notification when it is available on the website.

I just wanted to wrap up by saying thank you to all who participated in the call today. Again, a reminder about questions that we weren't able to answer live on today's call, that we'll be following up with each of you individually via email over the next week or two. We really appreciate everyone's engagement throughout the call and look forward to our next call in a couple of months. Thank you very much. Have a great day.