

Foundation Fighting Blindness
***Insights Forum* Transcript**
July 30, 2020

Chris Adams, Vice President, Marketing & Communications:

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

I would like to briefly review some logistical details for the call. Currently, all 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 today's event does not have any slides. Our speakers do have their video live, however, all their comments will be purely in an audio format.

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 like to turn the call over to Jason Menzo.

Jason Menzo, Chief Operating Officer:

Thank you, Chris, and thank you everyone for joining us today. We have a very exciting call planned. My name is Jason Menzo, and I am the Chief Operating Officer here at the Foundation Fighting Blindness.

I'd like to welcome you to our quarterly *Insights Forum* call, which is focused on highlighting the latest developments here at the Foundation Fighting Blindness and the broader inherited retinal disease community.

On today's call I will highlight some of our recent activities in the community including an update on our recent events. I will also provide a brief summary of our recent financial performance.

I am also very pleased to have Dr. Todd Durham joining us today. Todd is our Vice President of Clinical & Outcomes Research. He joined the Foundation in early 2019 in a new role that is focused on clinical research, such as natural history studies and the development of novel endpoints for inherited retinal diseases. Todd has been a great addition to our team, bringing strong expertise in biotech drug development and clinical trial design. He is going to provide an overview of our initiatives related to Natural History Studies. We often get questions about the purpose, funding and outcomes of these type of studies and we thought it would be a good idea today to give a forum for Todd to be able to explain some of the context around our efforts, what natural history studies are and why they're so important to help us meet our mission.

Then, our CEO, Dr. Ben Yerxa, will wrap up our formal remarks by highlighting our key accomplishments over the past fiscal year and positioning us for the year ahead.

Following our formal remarks, we'll be joined by Dr. Brian Mansfield, our EVP of Research, and Interim Chief Scientific Officer, who will be available to answer questions during the Q&A session.

At that time, Chris will provide the instructions on how to ask questions. As Chris mentioned a minute ago, this call is being closed captioned, and the replay and fully accessible 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 this call, or the Foundation in general, please reach out to us at the email address:

info@FightingBlindness.org.

Now let's start with a snapshot of recent activities and events that have taken place over the past few months and what's coming up here at the Foundation.

First off, let's talk about VISIONS 2020, which quickly became a totally virtual event we hosted at the end of June. VISIONS is our global conference that takes place every other year and brings together our global inherited retinal disease community.

I must say our community truly embraced the virtual platform for the conference this year, which led to significantly higher participation than we have ever seen before. We had more than 1,500 international participants registered from 45 different countries, plus we streamed all the sessions on Facebook Live and on our website to an even bigger audience.

Our attendees joined from all over the globe, spanning from Australia to Chile, the Netherlands and Ukraine to Egypt and Nigeria, and from Iran and India to Japan and Singapore. We truly had global participation. It was spectacular!

As an example, our opening session, which featured our CEO, Dr. Ben Yerxa, and leading researcher from the University of Florida, Dr. Shannon Boye. They kicked off the conference with nearly 4,000 people viewing the session on our Facebook Live stream alone. This speaks to the global nature of our mission and the thousands who have affinity to what we do.

The conference had an impressive array of topics and speakers, such as Advancing Cell Therapies, the Promise of Genetic Therapies, Mental Health and Isolation in our Current Environment and we even hosted breakout sessions to create a space for peer-to-peer dialogue in a social setting, along with our typical research updates on retinitis pigmentosa, Usher, LCA, Stargardt, and macular degeneration.

Overall, we were thrilled to see such a high level of engagement, both in the U.S. and internationally, and we look forward to our next Visions, which hopefully will be in person for the summer 2022 in Disneyworld in Florida! Of course, there will be more information on that in the months ahead.

One final reminder as it relates to Visions, audio and video replays for all of the Visions 2020 sessions are available at no charge on our website and can be accessed in the "Events" section.

Now let's move on to another key community focus for our Foundation - our VisionWalk program. In the fall of 2019, we hosted 17 walks which raised \$2.0 million towards our mission in those 17 walks alone. The remaining 19 walks scheduled to take place from this past April through June of 2020 – we had to pivot and we did. We shifted our approach and combined them into one National Virtual VisionWalk Day that was held on June 6, 2020. Again, we were humbled by the commitment and support that our community generated.

These Spring Virtual VisionWalk events raised more than \$1.3 million towards our mission. It is important context to keep in mind, \$1.3 million is an impressive number but it is only half of the revenue we typically expect for this slate of spring walks; however, on a percentage basis, it does outperform many of our peer organizations who have also pivoted to virtual walks this spring. It is important to note that the COVID pandemic has certainly impacted our fundraising and is forcing us to be creative as we go forward. I do want to highlight and thank our community as a whole, including our staff, our board members, our trustees, committees, volunteers, chapters and team captains from across the country who have stepped up to keep our mission moving forward despite the challenges we have been facing. I think it is important as we look months ahead that we continue to galvanize the community to help us because the environment unfortunately doesn't look like it is going to improve at least for several more months. I want to thank you but also ask for your continued support.

Building off the experience we had this spring, we are excited to announce our Fall National Virtual VisionWalk, which is scheduled for Saturday, October 24, 2020! Much like we did in the spring, we have a fun and exciting day planned as we celebrate our fundraising efforts towards our mission. And associated with that, we'll be hosting an informational kickoff session on Thursday, August 27, at 8:00 pm ET, with logistics and tips on fundraising for team captains and participants. You can find out more information and sign up to participate on our website homepage in the "Get Involved" section under VisionWalk. And much more information about the kickoff and VisionWalk will be sent via email and posted on our social media pages in the weeks ahead.

While most of our originally planned fundraising dinners, golf tournaments and other events for this summer and fall have been postponed or cancelled, we have a very exciting upcoming virtual event I want to share with you. It is open to everyone. We are excited and thrilled to announce this today. We're going to present a unique one night only livestream concert and conversation with Sam and Casey Harris of the rock band, X Ambassadors, a popular American rock band from Ithaca, NY. They have had five Billboard Hot 50 hits in recent years, including songs you may know, like Renegades and Unsteady. The band has a personal connection to our mission with keyboard player and founding member, Casey Harris, being blind from a rare genetic condition called Senior-Loken syndrome, causing him to be visually impaired from birth.

In the livestream, Casey, and his brother, Sam Harris (who is the lead singer/songwriter), will perform an acoustic set of their top hits. I'll have an opportunity to have a conversation with them to discuss their connection to our

mission – it’s really a fascinating and inspiring discussion that puts a personal face on one of the more prolific musicians in our community.

The event is being produced with support from our friends at *Two Blind Brothers*, the clothing company founded by Bradford and Bryan Manning which donates 100% of proceeds to retinal research.

It really does promise to be a huge event for us, and we’re hopefully going to introduce our mission to a whole new broad audience of folks out in the community and we are excited to bring it to you live in just a couple of weeks. We’ll announce the date and begin promoting the event in the next couple of days, so please stay tuned, and we’ll of course be emailing and posting it on social media and having a lot of awareness. I’m going to ask all of you to please share the date with your friends and try to get as many folks to participate and watch the live stream as possible. It will be free of charge. We’ll accept donations and hopefully we can raise some money in addition to raising awareness.

Shifting gears, there were a number of educational events that our professional outreach team have hosted virtually over the past few months.

We hosted Vision Seminars and Chapter Speaker webinars that have attracted more than 800 attendees.

In May, we hosted the Foundation’s first-ever continuing education webinar for eye care professionals. Dr. Jacque Duncan, who chairs our Scientific Advisory Board, provided an overview of inherited retinal disease, genetic testing, and clinical trials to more than 200 eye care professionals, many of whom were new to the Foundation. The recorded webinar is also now available on our website.

In June, we also hosted a genetic testing webinar-workshop for eye care professionals with our partners Blueprint Genetics and InformedDNA. The replay of this webinar is available at no charge for anyone interested and can be found on our website in the Genetic Testing section under Eye Care Professionals. Please note, you don’t have to be an eye care professional to listen and appreciate the information within these replays.

In Fiscal Year 2021, our professional outreach team is planning to participate in multiple virtual industry conferences and host additional continuing education courses and online webinars to maintain and grow relationships, especially with large eye care practices in metropolitan areas. We are also pursuing partnerships with biotech and pharmaceutical companies to expand our network and expand our reach.

Now let me provide a few highlights of our recent marketing and awareness building activities. We have been working hard to increase interactions with community

members and add additional content to our digital platforms. We have expanded the amount of content, including more personal stories, resources for the current COVID-19 pandemic, research news and sharing content from our other partners. All of this content is new and available on our website. And if you haven't gotten the theme from the first part of this call yet, our website is a great resource at www.FightingBlindness.org and it's constantly being updated with new content and information. We want to be a resource available to our community and I recommend checking back at our website every couple of weeks because there is always a new story, always new research news and it is a great place to stay up-to-date with what is happening in our community.

In particular, I encourage you to read some of the personal stories we refer to as our Beacon Stories, which are inspiring and uplifting stories about individuals in our community and their personal journey how their drive inspires them. They are found under the Newly Diagnosed Testing section of our website.

Our marketing team has also been focusing on optimizing our website to increase traffic from search engines like Google. Today, you will find the Foundation show up on page one for pretty much every search result on Google for keywords that are relevant for our mission. That was not always the case. Our team has worked really hard. When someone searches retinitis pigmentosa or inherited retinal disease, our website now shows up as one of the first items on Google, which is a really important endeavor to expand awareness of our mission, the Foundation and the resources available on our website.

In the past year alone, we have revamped our educational collateral, launched our Beacon Stories, and launched our public service advertising campaign which we have spoken about in previous calls. We have been very busy trying to get the word out and highlight our mission to the broad community.

Finally, I'm going to conclude my remarks with a brief summary of our financial position and performance. As a reminder, the Foundation Fighting Blindness operates on a fiscal year that runs from July to June, so we just completed our Fiscal Year 2020 on June 30. Our staff is working really hard to close the financials for the Fiscal Year, which as you would imagine is unlike any year we've ever experienced before with what is happening in the world today. The audited financial statements will be available later this fall and will be accessible from our website homepage in the About Us section under Financial Reporting.

We're able to share with you today the preliminary financial results for the Fiscal Year 2020, keeping in mind that these are just projections as the year-end close process remains in progress.

Our current projections for the year include total revenue of \$36 million, which includes \$5.1 million of investment income and a new \$15 million gift specifically for the RD Fund, which Ben will highlight later in the call today. Traditional annual fundraising efforts are projected to be significantly below our budget in large part due to the coronavirus pandemic. This revenue is against operating expenses of \$14.3 million, which includes public health and education, along with other expenses. We have been able to be very efficient and come in about \$1 million below budget on expenses. The net result is that we project the organization to be able to support about \$7 million in new research coming off of our performance this past year.

And I'm really proud to share that with the prudent financial management that we've been managing the organization under, we were able to award the full funds anticipated prior to the pandemic. We were able to fund all of our prior research commitments and fund new research commitments that Ben will highlight later in the call.

As we looked at 2021 and kick off our new fiscal year, our staff has continued to collaborate on ways to increase impact, to be as efficient as possible with the tightening resources we expect. We have worked with our Board to establish a Fiscal Year 2021 budget that includes funding all sorts of new research in accordance with our five-year strategic plan.

The key to the Foundation's sustainability and success is the commitment of community members like you. Your contributions in time, in talent and the resources that you provide us are essential in enabling us to continue funding critical research even during these challenging times. And on behalf of the entire Foundation, I can't thank you enough for your continued support. That concludes my section of the call today.

I would like to turn the call over to Dr. Todd Durham, our Vice President of Clinical & Outcomes Research. Todd, the floor is yours.

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

Thank you, Jason. I am pleased to have the opportunity to connect with you all on this call today.

I'm going to provide an overview of the purpose and impact of natural history studies and update you on the recent and ongoing initiatives we have in this important area.

There are several reasons that the Foundation funds and conducts these studies.

- First, it is critical that we understand the natural progression of disease. In dealing with inherited retinal diseases, or IRDs, there are nearly 300 different conditions related to genetic variations. These studies help us to evaluate how a disease progresses, estimate the rate of disease progression and how variable that progression is among groups of affected people.
- We also evaluate the usefulness of various outcome measures that help in designing future clinical trials for emerging therapies. An outcome measure is the result of a treatment or intervention that is used to objectively determine the baseline function of a patient at the beginning of the clinical trial. Once the treatment or intervention has commenced, the same measure can be used to determine progress and efficacy.
- The studies help us work with researchers to identify potential clinical trial participants in a confidential manner.
- In addition, we can make de-identified data from the studies accessible to researchers who can use it to design clinical trials.
- We also publish data from the studies in peer-reviewed publications that provide useful information for clinicians, patients and other community members to better understand the diseases.
- Our ultimate goal is to boost and accelerate the development of therapies, amongst both academic researchers and industry.

Over the past ten years, the Foundation has funded \$17 million to conduct natural history studies in several prominent IRDs, including Stargardt disease, Usher Syndrome and Retinitis Pigmentosa, or RP. Let me provide a brief summary of these studies.

- (1) In the ProgStar study, which was launched in 2013 and completed in 2017, we studied the progression of atrophy related to Stargardt Disease, which is a major cause of early macular degeneration.

- This two-year study prospectively followed 259 Stargardt disease patients and had a retrospective arm that enrolled 251 patients.
- To date, 22 study-related research papers have been published in peer-reviewed journals.
- For example, study results were recently published in the Journal of the American Medical Association's Ophthalmology online site. This study featured data that indicates that microperimetry may be a useful outcome measure for clinical trials of emerging Stargardt disease therapies. As many of you know, a microperimeter is a device that measures retinal sensitivity in the macula, the central region of the retina.
- Analyses of ProgStar data continue with additional publications in process.

(2) In the RUSH2A study, which was launched in 2017 and completed enrollment in 2019, we are studying retinal degeneration caused by mutations in the USH2A gene.

- This genetic mutation is a leading cause of Usher Syndrome Type 2A and autosomal recessive retinitis pigmentosa.
- This is an international, four-year study designed to better understand the time course of vision loss in people with USH2A mutations.
- We completed enrollment in this study in mid-2019, with approximately 125 patients enrolled at 20 sites in the US, Canada, and Europe.
- We have been following these patients and have begun to compile data to share with the community. There are currently several scientific publications from the RUSH2A baseline data that are in progress, so we will keep you informed on those results.

(3) In the Pro-EYS study, which was launched in late 2019 and is ongoing, we are studying retinal degeneration caused by a mutation in the EYS gene.

- The EYS gene is one of the more common causes of autosomal recessive RP.
- This is an international, four-year study evaluating a variety of clinical measures with the goal of identifying those measures that are most useful to apply in future clinical trials to show if a therapy is working.
- We have identified 30 sites to participate. We began enrolling participants in February 2020. Currently 6 sites have completed their certification requirements and 3 study participants are enrolled, with a target of a total of 100 patients.

As part of our current five-year strategic plan, we have targeted the funding and initiation of at least two additional natural history studies in the next several years. At least one of these natural history studies will seek to identify useful clinical endpoints and characteristics that are common across multiple retinal diseases, rather than the single disease focus.

These studies require a significant undertaking by the investigators, reading centers, laboratories, genetics experts, and study participants who make this essential work possible. We are truly grateful for these efforts.

In order to approach these studies in the most effective manner possible, we established the Foundation Fighting Blindness Clinical Consortium in 2016. This international network of clinical researchers works together using common protocols and data sharing to undertake important clinical studies. The consortium currently consists of nearly 40 clinical centers of excellence around the world – including the U.S., Canada, the U.K., France, Germany, Netherlands, Israel, Finland, and Poland. These centers have significant experience in IRDs and with standardized assessment protocols. The readiness and expertise of these centers helps speed up the often lengthy and expensive process of setting up and conducting clinical studies.

In February of this year, we hosted our first annual Consortium Meeting which included participation by nearly 50 clinical trial investigators and coordinators from nine countries. During the meeting, the investigators shared their data analyses on the RUSH2A natural history study; discussed the ongoing Pro-EYS natural history study and discussed future plans, including ideas for the next natural history studies.

In addition to these studies, we are planning other projects within our clinical research program. And we have interest from industry partners who share our strategic objectives. Some of the ideas under consideration are the following:

- We are interested in evaluations of patient reported outcome measures in individuals with IRDs. These can be part of our registry or as standalone projects.
- Studies of the impact of IRDs on daily living, professional life, and use of health care resources.
- And we are also interested in pursuing opportunities for affected individuals to participate in qualitative research.

We believe these initiatives will have numerous benefits for the IRD community. Our team at the Foundation sincerely appreciates all that each of you are doing to help fund, research, prevent, treat and potentially cure these blinding diseases. I'm now pleased to turn the call over to our CEO, Dr. Ben Yerxa.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you, Todd. Good afternoon everyone and thank you for joining our quarterly update call.

I'm going to wrap up our formal remarks today by highlighting our key accomplishments in the fiscal year that just ended in June and which position us well for the coming year.

We kicked off the previous fiscal year with a broad-reaching strategic plan to provide \$105 million dollars in funding over five years for critical scientific projects. Our multi-year plan encompasses the full spectrum of funding programs from early translational research to clinical studies, the fundraising and revenue required to fund the highest levels possible, and the communications and education plans that allow us to be as connected as possible with our constituents.

Our current portfolio of research projects includes approximately 80 grants conducted by research investigators at 75 institutions. In addition to funding researchers within the United States, our funding extends internationally to laboratories in Australia, Belgium, Brazil, Canada, England, Finland, France, Germany, Israel, Italy, Mexico, the Netherlands, and Poland.

I am pleased to share that despite the COVID pandemic, we are funding the entire portfolio of awards laid out in our plan for 2020.

Let me give you a summary of our new funding commitments in Fiscal Year 2020.

- We provided funding for individual investigators including six preclinical and two clinical research awards totaling \$2.4 million.
- We are funding three aspiring clinician-scientists interested in inherited retinal degenerations with one-year Clinical Research Fellowship awards totaling \$200,000 that will allow for post-residency clinical fellowships. The goal of this program is to increase the number of clinician-scientists with expertise and commitment to provide clinical care to patients with inherited retinal degenerations.
- We are also providing \$2.5 million over five years to the University of Pennsylvania Large Animal Model Translation and Research Center – this is the group who played a pivotal role in demonstrating safety and efficacy of the gene therapy Luxturna and who are continuing to make similar key contributions to other gene, cell and small molecule therapies that are in or moving towards the clinic.

- We awarded \$1.5 million to three research centers to develop new non-rodent large animal models for Stargardt disease, autosomal dominant retinitis pigmentosa and Usher syndrome type 1B. These are new a new category of awards introduced for the first time this year, that form part of the increased funding anticipated in our Five-Year Strategic Plan. These awards support the development of new animal models for diseases that lack appropriate models to support translation of research into the clinic.
- In addition, there is \$6.5 million targeted for the new Translational Research Acceleration Program (TRAP) that will provide increased funding support for research that is very promising. The goal is to accelerate particularly promising therapies towards the clinic, with increased funding and mentorship. This is a flexible program that supports academic researchers, as well as small biotech companies, where our funding can make a big impact.
- And finally, we are launching a call for applications to address early age-related macular degeneration research totaling up to \$600,000.

In selecting the recipients of these awards, we are guided by the review and input provided by our 50-member international Scientific Advisory Board, which is composed of the leading researchers in inherited retinal diseases.

Deploying a best-in-class governance model, the Advisory Board membership is structured with term limits to facilitate the addition of new and diverse members. In the past two years, we have added 17 new members, nine of whom are women.

We also created a new subcommittee of the Advisory Board, the AMD Scientific Advisory Subcommittee, to address expanding needs of the Foundation's research portfolio.

Our research award programs are particularly focused on the global support of new researchers, novel technologies and identifying new endpoints for clinical trials. We are also seeking therapies that go beyond treating a disease caused by a mutation in a single gene, to develop single therapies that will treat multiple different genetic diseases – often referred to as pan-disease therapies.

Our Board of Directors mandates that at least 25% of new awards are from investigators new to the Foundation and we have consistently met this requirement and often exceeded the goal.

Of the 15 new projects, nearly 50% are led by investigators new to the Foundation and more than one-third were awarded to researchers outside of the U.S.

You can find more information about these awards from our website homepage in the Research section under the Scientific Grants and Awards Programs link.

Another key part of the Foundation's opportunity to advance treatments for IRDs is our Retinal Degeneration Fund, what we call the RD Fund, which invests in companies with projects that can be in clinical testing in 18 to 24 months.

The Fund has invested in multiple companies working on a range of promising technologies and therapeutic targets.

- CheckedUp is a healthcare IT company that provides scientific and medical content to large screens in physicians' waiting and exam rooms, and they've recently launched a new telemedicine platform in the midst of the pandemic.
- Nacuity just started a Phase 2 clinical trial in Australia for a breakthrough treatment for RP associated with Usher Syndrome.
- Nayan Therapeutics is developing mutation-agnostic therapies to treat inherited retinal diseases.
- ProQR, who you heard about last time on the call, has a Phase 2 clinical program for Usher syndrome type 2A and is developing other treatments for severe genetic rare diseases such as Leber's congenital amaurosis 10 and autosomal dominant RP.
- SparingVision is developing an emerging, cross-cutting gene therapy designed to preserve cone photoreceptors, thereby saving vision, in people with many forms of RP.
- Stargazer is wrapping up Phase 1 clinical testing for an oral, nonretinoid visual cycle modulator to treat Stargardt disease.
- Vedere Bio is developing next generation optogenetic gene therapies which are a pan-retinal approaches to restore vision in eyes with little to no remaining photoreceptors.
- And our most recent investment is with Atsena Therapeutics. Atsena is a startup gene therapy company coming out of the University of Florida and Shannon Boye's lab that was just announced yesterday. They are developing novel gene therapies for IRDs, including a Phase 1/2 program for LCA1 that was licensed back from Sanofi.

We are now about 75% committed for our first fund. I said "first" because we continue to experience high deal flow, with many exciting opportunities that need more capital. I'm very excited to announce that, based on the success of our initial fund, the RD Fund

Board and the Foundation has approved an investment and fundraising plan for a second fund, called RD Fund 2. We are extremely grateful to have a foundational commitment of \$15 million for the new fund from the Manning Family Foundation. This provides tremendous momentum for our second fund and will enable significant investments in ground-breaking programs. You can find more information online at www.RDFund.org.

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. Over the past year, we have made remarkable progress in expanding the My Retina Tracker Registry and the related open-access genetic testing program, increasing the number of Registry participants by more than 25% to 16,200 members currently.

Last October, we launched a new nationwide program that allows any clinician able to diagnose an inherited retinal disease to order a free, comprehensive, genetic test and genetic counseling, for their patients.

We now have genetic testing information provided by nearly 8,000 members and our goal is to have 40,000 Registry members by 2024, including more than 20,000 with genotype information available.

I'd like to wrap up by sharing several comments related to our Visions Conference that remind us why we are all working so hard to achieve our mission to drive the research leading to preventions, treatments and cures for degenerative retinal diseases.

- One participant said: "The Mix and Mingle was an amazingly helpful session and I wish we could do something like this more often, and not just when the entire world is virtual. Attendees shared what apps on their phones help them with daily activities and I think everyone really benefitted from hearing that info."
- Another participant said: "I loved meeting other people who are going through what I am and understand my disease. When you connect with other visually impaired people at an event like this, it makes you realize you're not alone." She also said, "I've been fundraising so long, and I knew it was going toward research, but to actually learn and hear about what science is doing makes me feel so hopeful."

While there are clearly economic and healthcare challenges in today's COVID-impacted environment, we continue to have tremendous optimism for the breadth and potential of research happening across academia and industry.

With your support, we are committed to keeping the momentum going with continued robust and expanded research funding and meaningful community engagement. Thank you so much.

Jason Menzo, Chief Operating Officer

Thank you, Ben. It is truly an exciting time for the Foundation as we move into our 50th year as an organization. It is about 1:40 pm ET here on the East Coast. We have about 20 minutes reserved for Q&A, which is terrific. I'm going to ask Chris to please provide the instructions for asking questions again.

Chris Adams, Vice President, Marketing & Communications:

As a reminder there are several methods for asking questions.

- First, you may access the Q and A and Chat features located at the bottom of the Zoom control bar and type in your questions.
- Second, you can ask questions verbally. To do so, please select the hand raising function on the menu bar at the bottom of the Zoom interface and we will provide you with instructions to unmute yourself.
- And third, if you joined by phone for today's call, you can press Star 9, to raise your hand. Pressing Star 6 will mute and unmute your line. You may also submit your questions via email at info@fightingblindness.org.

Please note that if there are questions that we aren't able to answer on today's call due to time constraints, we will follow up with you directly via email over the next week or two.

Jason Menzo, Chief Operating Officer

Thank you so much, Chris. And while we are compiling questions, I want to take a moment and thank everyone again for participating today and remind you that there will be a transcript and audio recording of today's call on our website within the next week.

In addition, we would like to ask for your help. Due to the COVID-19 pandemic, many of the clinical trials or lab studies have been delayed or canceled, including those for sight-saving treatments and cures for retinal diseases. There is new legislation that has been introduced in Congress to provide supplemental grants to federally funded

research programs, including \$15.5 billion for projects at the National Institutes of Health and the National Eye Institute. Researchers need this funding to get critical projects back on track.

While the Senate has agreed to this funding level for supplemental funding, the House has yet to do so. You can urge your House representative to support these supplemental research grants by visiting the non-profit website, www.ResearchAmerica.org, under the Contact Congress page. In less than a minute, you can send a letter to your representatives to let them know how important this legislation is to all patients and families across our nation that are affected by the current situation. With that, let's take our first question. Chris, what do we have to kick off our Q&A session?

Q&A

Chris Adams, Vice President, Marketing & Communications:

Our first question ties into the comments Jason just made about the status of ongoing research. Todd, can you comment on how COVID-19 is impacting clinical trials and your natural history studies?

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

Thank you for the question. Certainly, there has been an impact of the coronavirus on our clinical studies. There is a bit of a difference between when the pandemic began in early March and where we are today. Early on, the research centers were quite restrictive in who they can see. Now we've seen a number of centers involved in our study are able to see study participants again. Some still have some restrictions. The priority has been given to individuals who participate in clinical trials of an interventional therapy because there are important safety reasons why you need to be seen on time by your research physician. Those studies get priority. We are also seeing a number of our centers who are participating in our natural history studies that are open and are enrolling patients in our live study that is open for enrollment but not all centers are yet open depending on where they are in the country and the status at their respective institutions.

One thing I want to add about this, too, we have been sharing information amongst the investigators in our network. They have shared information about best practices how

to manage patient flow to keep a safe distance in their waiting rooms. We gathered information from the manufacturers who make the equipment to do the imaging for the studies and shared those clinical protocols across the network. I'm quite reassured our investigators are doing everything they can to make their facilities as safe as possible, so folks feel comfortable coming back in to participate in their research studies. Thank you for the question.

Jason Menzo, Chief Operating Officer

Thank you so much, Todd. And thank you to everyone who submitted questions so far. We will get to many of these questions here on the call. However, for those questions that we don't get to, we will follow up with each of you individually to make sure that all questions that are asked in the session get appropriate answers. The next question I'm going to direct to Dr. Brian Mansfield who joined us for the Q&A session. At the end of the formal remarks, we referenced all the wonderful new grants and awards that were awarded as we ended fiscal year 2020. From the time when the grants are awarded to the time they show up on the Foundation website under the Research section, what is taking place now and when will the public find out about what those awards are and when will that formal announcement take place.

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

There is always a time difference when the board votes to approve an award and when they appear in public. There are a series of administrative steps that we need to go through after the award is approved. This is where we reach out to the universities and we work with their contract offices to make sure that the conditions of the award are appropriate and they agree to our terms and financial reporting on the way we request scientific updates on a regular basis. There is a little bit of paperwork that goes through that process. When that is finally completed across all the sites that we have made awards to, then we publish the summaries of those awards on our web page for everyone to read about. This can take one or two months, depending on the speed in which we need just to process the agreements and make sure everything is in place appropriately.

Jason Menzo, Chief Operating Officer

So today everyone got a quick preview of the information, which is terrific and we're happy to share obviously. I'm going to direct this next question again to you, Brian. Several questions around the announcement that Ben mentioned that we made in October that any ophthalmologist or eye care professional can diagnose IRD and get their patient access to genetic testing. There are a couple of questions I'm going to

combine here but if you could speak to the types of eye care professionals who can make a clinical diagnosis of an ERD, what that task might look like for a patient and what that process might look like for ordering our free genetic testing to our program.

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

As part of our open access genetics testing program, anyone who is clinically diagnosed with an inherited retinal disease, who lives in the U.S., is eligible for testing as long as they have not had a comprehensive gene panel test since 2016. If you have had anything more than 32 genes that are related to an inherited retinal condition, tested since 2016, you would not be eligible just yet because your testing is too new. If you haven't had one in a while, then you certainly are eligible. The eligibility of clinicians who are able to order the test vary state by state. If you're under the care of an ophthalmologist or retinal specialist, then that is fairly straightforward. They're allowed to order a clinical diagnostic test through Blueprint. If you're under the care of an optometrist, it becomes slightly more complicated. Different states have different regulations about whether or not an optometrist may order a diagnostic test or not. It depends on if you are under the care of an optometrist and you would like them to order the test, you need to ask them if in your state they are allowed to order the test or not. It is only a small number of states that don't allow it but again, the testing laboratory that runs the test has to respect the state regulations. Once you have determined if you're eligible to have the test taken all you need to do is ask your clinician to order the test on your behalf. If they are not familiar how to do that, they can go to our web page, www.fightingblindness.org, and under there you find the tab for Genetic Testing. If they click on that tab, there is information about it. There are two downloads which are important. One is a PDF document for the person who is being tested, explaining what they will need to do and what the test will provide them. And the other PDF is a download for the doctor which tells them how to go about ordering the test from Blueprint Genetics. It is important to remember that only a clinician can order the test on your behalf. You are welcome to download that document for the clinician. In fact, you might even want to take it with you when you go for a visit to ask them to order the test. But a patient cannot order the test. It has to be done by a clinician. And again, these are regulations that the testing laboratory have to adhere to. I hope that answers the question.

Jason Menzo, Chief Operating Officer

Thank you so much, Brian. We've had a number of questions related to the impact of COVID 19 on our community both as it relates to clinical trial enrollment and also whether or not there is any documented or initial overlap between the impact of

COVID 19 specifically. The question is referencing from a pathophysiology standpoint and IRD community. Todd, I think you're best to speak to COVID 19 and you spoke to it a little bit ago. The question has come up before about whether there is any mechanistic overlap between COVID and IRD.

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

The current state of play here, at least in the United States, is that some centers are open and conducting research as before. Again, with some additional safeguards with respect to cleaning equipment and appropriate spacing or more distance spacing in the waiting room. Other centers around the world, such as in Europe, are more like we were in the first of the year and don't have as many restrictions. It really depends on where you are in participating in your particular clinical trial. But one message I have heard many, many times is that all centers are being particularly careful to have appropriate distance and to reassure study participants that their environment is as safe as possible.

Jason Menzo, Chief Operating Officer

Ben, do you want to speak to anything in the literature, anything in the scientific community as it relates to anything documenting an overlap between COVID 19 and direct impact on the IRD community?

Dr. Ben Yerxa, Chief Executive Officer:

I'm not sure I have a good answer for that to be honest with you. We're all just trying to figure this out on the fly.

Jason Menzo, Chief Operating Officer

I know that question came up on another call yesterday. The state of play is that we're all trying to figure it out and that the short answer is there is nothing that is published to show overlap, but you know it is so new. It is hard to see where it is going.

Brian, there is a question around whether or not the free genetic testing program is only eligible to patients that are enrolling in a clinical trial supported by the Foundation Fighting Blindness. The answer is that it's available for any patient diagnosed with an IRD eye care professional but maybe you can speak to the role that genetic testing can help in accelerating folks getting enrolled in clinical trials. That question always comes up often and has come up a couple of times today as well.

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

You don't have to be in a clinical trial to have open access testing. You have to meet the criteria I mentioned previously and have a clinician order the test for you, as long as you live in the U.S. Now, unfortunately we can't help people who are living outside of the U.S. at the moment. Although if your clinician contacts me if you're outside the U.S., I may be able to have you get access to discounted testing. Genetic testing is really important these days because a lot of the therapies that are in the clinical pipeline are based around treatments for a specific gene and when you look at some of the therapies like that being developed by ProQR, they can be to a specific mutation within a specific gene. It is increasingly important these days to understand the genetic cause of your disease if you can and that is what a genetic test will help you understand.

I should however comment that while a lot of people are excited by genetic testing, there will be a group of people currently about 50% of people who go through a genetic test who will not get a clear explanation of the gene causing their disease. Now, these are often people who have an autosomal recessive disease like autosomal recessive RP because the success rate of finding the genetic cause of disease is by the mode of inheritance of the disease. But, on the other hand, many people will get an informative result. I think it is very important that if you don't know the genetic cause of your disease, that you seek out a genetic test and moreover, when you do that, we really would appreciate it if you would join My Retina Tracker Registry, if you're not already a member, and enter that information into the Registry so that information can help accelerate treatments and cures for all of us.

Jason Menzo, Chief Operating Officer

Thank you, Brian. Maybe one follow up question on genetic testing and then a question for Todd on prep on the EYS gene. Brian, first, some of the questions I'm trying to put into one theme here for you which is around the advancements and accuracy or important information that is able to be identified out of genetic testing today as compared to perhaps someone that had a genetic test several years ago. Maybe you could speak a little bit to that because I know some folks have been deterred if they have had a genetic test many years ago because they didn't get the answers they were seeking and how their expectations could be managed with what they could expect today with the advancements and technology.

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

There are two aspect to that question. The first is that many people who were tested a while ago have expressed frustration to us at the Foundation that they never heard a clear result back. The reason for that is there are two different types of genetic testing that can be undertaken. And until we started our Open Access program, a lot of people

were receiving a test which would not guarantee a result back. Let me explain the difference here.

If you went for a genetic test and they did a test under what are called CLIA regulations, these are validated tests where it is agreed if “you don't find a cause of the disease, you may report that I have not found the cause of disease”. If that happens, you are probably offered another genetic test which would fall under the title of a research genetic test. Now, research genetic tests are done by individual researchers who have not had their tests approved to CLIA standards. And so while they will seek to find the genetic cause of your disease, they may not find it. It may be that your gene wasn't known and had yet to be discovered, but they couldn't actually tell you, we haven't got that information yet because the tests are not approved.

So many people ended up in a situation where they were not hearing back and getting upset. And the reason was that your result from a CLIA test had not been useful and the second test not being CLIA certified means that the results could not be reported back to you.

The way to ensure that you always get a result back is to have a CLIA certified test and in the Open Access program and for those of you who are aware of our other program called the IRB program, both of those use a CLIA genetic test and therefore you will hear back whether we find results or not. You should hear back within about four to six weeks about the result.

The other reason that you may not get clarity on the genetic cause of disease comes into the subtlety of genetics. Genetics does not give a plus and minus result. If you have it or if you haven't got it. There is also a third category of “we're really not certain”. The differences between your gene and my gene may just be because we're different people. Maybe because I have got a disease and you don't or because you have got a disease and I don't. And the challenge is to differentiate mutations that that cause the difference just between you and me and our size, our skin color, our hair color, our height, our body weight, those kind of things. Those that cause disease. These are called variance of uncertain significance. We just don't know the significance of that variation or that mutation. And so when you undertake a genetic test you can sometimes get a test that says yes. That mutation we definitely know causes a disease. Then you can get one where we say no. We're absolutely certain. That is just because you and I are different. And then there are variants we find we just don't have enough information about at the moment that say there is something different in this gene that could cause a disease, but we don't have enough information. We haven't seen this in enough people who have the disease to be able to say that this is definitively the cause

of your disease.

So sometimes you get this third category of uncertainty in a diagnosis. This is why genetic counseling is really critical when you have a genetic test. Part of our open access genetic testing program is to provide you with a no cost genetic counseling session. In genetic counseling a person who is experienced in the clinical presentation of the disease and who also understands the complexity of genetics is going to look at your result and interpret it for you and explain to you in very simple language what I have been trying to shortly summarize so far. So they will explain to you the limits of your test, the certainty of it and they'll also tell you about therapeutic options, what clinical trials are relevant to you, what to look out for and the research literature. The clinical counseling is really important to help you understand your test and it will help explain these variations that you'll be getting in your genetic test reports.

Jason Menzo, Chief Operating Officer

Thank you so much, Brian, for that detailed overview. It is just a minute after 2:00 so we're going to take two final questions that I have queued up here. One I will direct to Todd and then one we'll send to Ben. Todd, a couple of folks have asked about the prevalence of the EYS mutation, specifically in the U.S. I wonder if you could address that. Then we'll wrap up Ben with you speaking to a couple of highlights that are happening in the world of Usher Syndrome.

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

I'll just briefly respond and then ask Brian to supplement my response. The percent of patients with autosomal recessive RP attributable to mutations in EYS gene ranges across the world from 7% on the low side to 24% on the high side in Japan. It is quite a bit of geographic variation. The information that I have in the study protocol doesn't reference a particular result in the U.S. I believe Brian has some additional information.

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

Thank you, Todd. In the genetic testing study that is available to everyone, we have taken around seven and a half thousand genetic tests and are starting to build data around this. In our testing program, EYS is the 6th most common genetic cause of an inherited retinal disease. The most common genetic cause of disease we have found in our data is Stargardt disease caused by mutations in the gene ABCA4; that is followed by the USH2A gene which causes Usher Syndrome and autosomal recessive RP; then the RPGR gene which causes X-linked RP; then a form of autosomal dominant RP due to PRPH2 gene mutation; followed by another autosomal dominant RP due to Rhodopsin

gene mutation; and sixth in line is the EYS gene. Currently, our numbers suggest that those that have been tested and had a clear diagnostic result, about 3% of people with an inherited retinal disease in the U.S. have the EYS gene. Which means that in our data, it represents about 6% of RP cases.

Jason Menzo, Chief Operating Officer

Thank you, Brian. Finally, Ben, can you just speak to a couple of highlights that are happening in the world of Usher Syndrome as it relates to trials.

Dr. Ben Yerxa, Chief Executive Officer:

In terms of actual clinical trials that are ongoing right now, as we mentioned ProQR has an Usher syndrome type 2A trial that is ongoing right now, targeting mutations in exon 13 of the causative USH2A gene. And, then, Nacuity just started a Phase 2 clinical trial in Australia for a breakthrough treatment for RP associated with Usher Syndrome. Those are the two I'm aware of right now. And there are certainly several in the preclinical pipeline, so stay tuned over the next year or so.

Jason Menzo, Chief Operating Officer

Thank you so much, Ben and it is about five minutes after 2:00 here on the East Coast. We're going to wrap things up. I do want to say thank you to all of our participants on today's call. And for anyone who asked a question that we were not able to get to live on today's call, as I mentioned earlier, we will follow up with you via e mail over the next couple of weeks. If there is any other information that you need or any other questions or suggestions that you have, please feel free to email us at info@fightingblindness.org. And of course, our Foundation website is www.FightingBlindness.org. We referenced the RD fund several times in this call. The RD fund website is www.RDfund.org. Again, we really appreciate everyone's engagement throughout the call and look forward to our next Insights Forum call. Also, we'll provide updates via email to all the events upcoming that we spoke about earlier in the call and please stay tuned for those. We'll be in touch again soon. Thank you everyone and have a great rest of the day. That concludes today's call.