Foundation Fighting Blindness
***Insights Forum* Transcript**
April 23, 2020

Chris Adams, Vice President, Marketing & Communications:

Good afternoon and welcome to the Foundation Fighting Blindness Insights Forum call. I'm Chris Adams, Vice President of Marketing and Communication 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 available in closed captioning. To activate the closed captioning, please select closed captioning at the bottom of the interface. Today's event does not have any slides.

If you are using a screen reader, please be aware the controls are at the bottom of the Zoom interface. This control bar may collapse when not in use. If you prefer to prevent the controls from auto hiding, go to the settings within the Zoom platform, select accessibility and then select always show meeting controls.

To ask questions during the event, you may use one of three methods. First, you may access the Q&A feature on the Zoom control bar to type in your questions. Second, you may ask questions verbally. To do so select on the hand raising function on the menu bar at the bottom of the Zoom interface and we will provide instructions on unmuting yourself. Third, if you joined in by telephone only and not on the Zoom app, please submit the questions via email to info@FightingBlindness.org. At this time, I'd like to turn conference over to Jason Menzo.

Jason Menzo, Chief Operating Officer:

Thank you, Chris, and good afternoon, everyone. Thank you for joining us today. Hope everyone is staying safe and healthy as we navigate the unprecedented times in this challenging global situation. My name is Jason Menzo and I'm the Foundation Fighting Blindness Chief Operating Officer and I would like to welcome you all to the Insights Forum call. The purpose of the call is to highlight the latest Foundation developments and the broader inherited retinal disease community. We have a packed agenda for you today. First, I will be providing an update on the impact of the COVID related pandemic and our related response. Then I will highlight recent activities to increase community engagement and public awareness of our mission. I will then wrap up with a summary of our most recent financial performance. Next our Executive Vice President of Research and Interim Chief Scientific Officer, Brian Mansfield, will provide an overview of science and clinical developments. And also we have a special feature

today. We are very excited to have ProQR CEO, Daniel de Boer, as our featured guest speaker. Ben Yerxa will introduce Daniel who will share a summary of the company's recent news. Following our formal remarks, we will have a question and answer period and at that time Chris will repeat the instructions on how to ask your questions. As Chris mentioned, this call is being closed captioned and a 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 e mail address info@FightingBlindness.org.

Now, let's turn our attention to the COVID-19 pandemic and how it is affecting us here at the Foundation. The world is continuing to change rapidly and we are all dealing with the crisis on a global basis. Here at the Foundation, we are monitoring the evolving situation minute by minute and focusing on making the best possible decisions with the health and safety of the blindness communities and families.

Let me provide a specific update. Currently the Foundation is fully operational and this is actually our 6th week with our staff working remotely. While many of our business processes have changed, the team is adapting and really has pivoted to this new reality very well under the circumstances. However, given the broad state and local stay at home orders, most of our near-term events have been postponed or canceled or will be pivoted to a virtual type of format. This includes our Investing for the Cures Summit, which was postponed from its original date back in March. We have also shifted to holding several public health events in a virtual setting such as our recent Visions webinar series.

Earlier this month we made the very difficult decision to pivot our approach for Visions 2020, which is our global inherited retinal disease conference that was planned for June in Minneapolis. We plan to shift Visions 2020 to a new approach that will still allow us to meet but not in person. Our team is actively developing a virtual experience that will provide a wide range of science and research presentations and practical sessions on adapting and thriving with vision loss and an opportunity to collect with others across the country but in a virtual way. Please stay tuned as we share more information about our new VISIONS 2020 in the coming weeks.

Another staple in the spring calendar is shifting this year. Since we are unable to gather in person for the spring VisionWalks, we will have a virtual VisionWalk experience. We challenge everyone to celebrate with us on June 6th, for our National Virtual VisionWalk Day. After all, we are stronger together as a community and encourage everyone to get together and demonstrate the strength as a community during these difficult times. Please go to our website at info@FightingBlindness.org to learn more and to register

yourself and register a team to participate in our National Virtual VisionWalk Day on June 6th, 2020.

On Tuesday, May 5th, we are also excited to participate in Giving Tuesday Now, which is a global day of unity as a response to the unprecedented need caused by COVID 19. Together, we can stand united as we give back. The Foundation appreciates all of you in the community - because of you, we are making a difference in advancing our mission. So there will be a very active social media campaign on Tuesday, May 5th to bring awareness and fundraising for our mission at the Foundation.

We are also very focused on providing as many online resources as possible to our community during this time. Our website info@FightingBlindness.org, FaceBook page, Twitter, LinkedIn and Instagram accounts are all great sources of current news and information. If you have questions about any specific condition or current research you can always find detailed information on our website at the Newly Diagnosed or Retinal Diseases sections. For more information about genetic testing and the My Retina Tracker Registry, visit our new Genetic Testing section on the website. Of course, you can always reach us directly by sending an email at info@FightingBlindness.org.

As you could imagine during these unprecedented times, there will be significant impact on our fundraising but I want you all to know that we continue to prudently manage the Foundation's resources and are prepared to adjust as the situation dictates in the months ahead.

Despite these challenging circumstances, we have continued to make tremendous progress building community engagement and public awareness. A key part of our mission is to help make connections between various stakeholders in our community.

Our Professional Outreach team continues to make strong connections with the eye care professionals and groups serving low vision community. Through the hard work of our team, we have had the opportunity to share the Foundation's mission and activities with several key organizations serving our community, such as American Academy of Optometry, American Academy of Ophthalmology and ARVO and American Council of the Blind to name a few. We also continue to feature inspiring stories about members of our community in our Beacon Stories initiative, which is funded in part by support from the Allergan Foundation. We are excited to share more of these stories, some of which include video interviews, in the weeks and months ahead. These stories are feature on our website and social media channels.

We are also hosting an online continuing medical education webinar in May which will be delivered by Dr. Jacque Duncan who is Professor of Clinical Ophthalmology at UC San

Francisco. Dr. Duncan serves as chairperson of the Scientific Advisory Board. This is a new initiative for us that we are really excited about to be able to provide continuing medical education to the eye care community.

In terms of leadership within the community, we continue to enhance our engagement with our National Trustees, who are leadership level volunteers supporting the Foundation's fundraising, organizational development and volunteer recruitment efforts. We have begun hosting regular quarterly touch point calls led by Scott Burt, who's the Trustee representative to our Board. The goal is to keep our National Trustees informed and connected with all that's going on from a strategic perspective with the Foundation. If you are interested in learning more about the Trustee program, please send an email to info@FightingBlindness.org.

We continue to have success with our Public Service Announcement (PSA) campaign. This PSA campaign to date has received by over 692 million impressions which equates to over $15.8 million worth of media value. Again, a Public Service Announcement like this provides media exposure that comes at no cost to the Foundation. So that's incredible.

I would like to wrap up my section here today by providing a brief summary of our financial position. The Foundation operates on fiscal year that goes from July through June so our 2020 fiscal year will be coming to an end on June 30th, 2020. As of March 31st 2020, so through three quarters of our year, our actual year to date revenue was approximately $16.9 million against operating expenses of $10.2 million. Prior to the COVID 19 pandemic, we were on track to achieving our budget, however, things are evolving day by day and we are developing new projections based on the pandemic. It is safe to say given the economic impact of the crisis, we can expect the full fiscal year revenue to miss the original budget. But with that said, we still expect to fund all the prior research grant and award commitments and still expect to achieve a net surplus for new research commitments going into the next fiscal year.

Our team at the Foundation has rapidly taken action to adjust expenses as much as possible while still maintaining a high level of service and support that we provide to the community. We appreciate all of your contributions in time, talent and resources to help us fund critical research during this challenging time. Now I would like the turn the call over to Dr. Brian Mansfield, our Interim Chief Scientific Officer.

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

Thank you very much, Jason. I'm pleased to have the opportunity to provide an update on the many exciting clinical and research developments happening in the area of

retinal disease. ;

The Foundation continues to implement our five year strategic plan initiatives. One key focus for that at present is the annual call for applications for research project funding. This year it includes continuation of our core award programs. They are new career development awards, new individual investigator research awards, new translational research acceleration awards, or TRAP awards, which are for research with particular promise that we would like to accelerate toward clinical trials; and new awards specifically for clinical innovation.

I'm also pleased to share despite the COVID-19 pandemic, we are fulfilling the entire portfolio of awards laid out in our strategic plan. This not only includes the awards I just mentioned, but it also includes additional funding for brand new programs to further expand our work. These new programs include: the Free Family AMD Award to support research into age-related macular degeneration or AMD, awards to support the development of more clinically relevant animal models of the inherited retinal diseases and award to create a public database to help increase the understanding of the genes and mutations that cause the inherited retinal diseases.

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 - a truly international portfolio of programs.

Another key resource for Foundation support is Foundation Fighting Blindness Clinical Consortium. This is an international network of clinical researchers who work together using common protocols and data sharing to undertake important clinical studies. The consortium consists of 38 clinical centers of excellence around the world, including the U.S., Canada, U.K., France, Germany, Netherlands, Israel, Finland and Poland. In such a large network, strong communication is critical and one of many interactions that are held in person meetings.

In February, we hosted our first annual Consortium meeting of this year in Dallas. Nearly 50 clinical trial investigators and coordinators from nine countries participated with Foundation staff starting this meeting. During the meeting, the investigators shared their data analyses on the RUSH2A study and discussed the newly launched Pro-EYS study and future plans, including ideas for the next natural history study to be undertaken. Let me share with you a summary of the progress on the two ongoing natural history studies which are critical for understanding the impact any therapy may

have on the natural progression of disease.

First, I would like to talk about the Pro EYS natural history study. It is called the Rate of Progression in EYS gene Related Retinal Degeneration. The EYS gene is one of the major causes of autosomal recessive retinitis pigmentosa. This four-year study is 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 or not.

Our goal is to boost and accelerate the development of therapies to treat retinitis pigmentosa due to EYS mutations, amongst both academic researchers and industry. One way we do this it is to make de-identified data from the study accessible to all researchers who can use it to design clinical trials.

There are now 33 participating sites in the study that are in various stages of being certified. We enrolled the first participants in February of this year and are targeting a total enrollment of 100 people. For an up to date list of participating sites, individuals who have retinal dystrophy caused by the EYS gene and who are interested in participating in the Pro EYS study, should contact the Jaeb Center by email at FFB@JAEB.org for more information. This study is a significant undertaking and we are grateful to the investigators, reading centers, laboratories, genetic experts and study participants who are making this possible.

Next, I would like to talk about our ongoing RUSH2A natural history study. In 2017, we launched a study named Rate of Progression in USH2A-related Retinal Degeneration for people with mutations in the USH2A gene, which is a leading cause of Usher Syndrome type 2A and another form of retinitis pigmentosa.

A major goal of the four-year study is to better understand the time course of vision loss in people with USH2A mutations, so that researchers can design successful clinical trials for potential therapies and identify patients appropriate for the treatment studies. In mid-2019, we completed enrollment with approximately 125 patients across 20 sites in the U.S., 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 being prepared, so there's more to come on that front in the near future. You will hear more about progress in developing treatments for disease caused by USH2A in Daniel's remarks later.

Now I would like to move on to talk about My Retina Tracker Registry. This is a key initiative that supports the development of treatments and cures for inherited retinal

diseases. It does this by collecting information about how common each of the different inherited retinal diseases are, who is affected by those diseases, how it affects their lives, how common specific genes and mutations are in causing the diseases and synthesizing this to provide a database and a single point of contact where you can find defined groups of patients when there's a study or clinical trial. This is very important in accelerating our field. Our diseases are rare so it is hard to find people unless you know where they are and this is what the Registry enables us to do quickly and efficiently. Without it we can see research and clinical trials slowed and significant costs added.

My Retina Tracker Registry overcomes that barrier and joining the Registry is the one thing that any person with inherited retinal disease can do, at no cost, to raise awareness of their particular gene and condition and help accelerate our mission. To date, the Registry has over 15,500 people affected with the disease registered and it is most comprehensive international patient database for individuals with an inherited retinal disease. More than 7,000 genetic test reports related to these patients are also included in our database, making it very valuable.

I would like to turn to genetic testing. An essential companion to the Registry initiative is the Foundation's genetic testing program, which is gaining significant interest and traction from eye care professionals. This is a nationwide program launched this past fall. It allows any clinician who has diagnosed an individual with an inherited retinal disease to order a high quality comprehensive genetic test along with genetic counseling. Now, there's no cost to either the clinician or the person being tested. Any person with an inherited retinal disease who lives within the U.S. and U.S. territories may be eligible. This program provides people with inherited retinal disease easy access to their genetic diagnosis and facilitates therapeutic development by identifying and accelerating the enrollment of eligible people into studies and clinical trials.

This program is proving very successful. Half of all people in My Retina Tracker Registry, about 7,500 of the 15,500 patients have participated in this program already. The Foundation has conducted multiple educational seminars with doctors and eye care clinics to educate them on the program and guide them how they can enroll their patients into the genetic testing program. You do not have to be a member of the Registry to participate but we appreciate everyone that joins and shares their data to help accelerate the research.

We are really pleased to offer this free genetic testing program, which is made possible through generous support of the George Gund Foundation, Sofia Sees Hope, AGTC, Eloxx Pharmaceuticals, Kala Pharmaceuticals and MeiraGTx, along with ProQR.

This afternoon, I'm going to include my remarks with a brief mention of recent progress in ongoing clinical trials in IRD research. The large pharmaceutical company, Biogen, which acquired Nightstar Therapeutics last year, announced initial Phase 1/2 trial results for gene therapy to treat X-linked retinitis pigmentosa (XLRP) caused by mutations in the gene RPGR. Allergan and their partner, Editas Medicines, have dosed the first patient in a ground-breaking Phase 1/2 trial using the first in human gene editing therapy. They are using the CRISPR/Cas9 editing system to overcome genetic defects in the CEP290 gene and we are excited to watch the progress of this new therapy. The company, Horama, licensed rights to their gene therapy program for mutations in the CRB1 gene from Foundation-supported researchers at Leiden University Medical Center in the Netherlands. ProQR announced interim analysis for its Phase 1/2 trial of QR-421a for Usher Syndrome. You will hear more about these results from Daniel shortly.

In summary, we are very excited about the breadth and potential of research happening across academia industry. Our team at the Foundation sincerely appreciates everyone in the IRD community for all that you are doing to help prevent, treat and potentially cure these blinding diseases. Thank you. I'm pleased to turn the call over to our CEO, Ben Yerxa.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you Brian. Good afternoon and thank you for joining us on our quarterly update call.

Collaborations for academic and corporate organizations are critical to fulfilling the mission of the Foundation to drive the search for prevention, treatments and cures for inherited retinal diseases. Over the past several years, we have developed a strong and important partnership with ProQR and there are several key aspects to the relationship.

First, in 2018, we entered into a partnership to develop QR-421a for Usher Syndrome type 2A, targeting mutations in exon 13 of the causative USH2A gene. The Foundation's RD Fund committed to providing milestone-based co-funding of up to $7.5 million to ProQR to advance this program into the clinic. The most recent expansion of our partnership was announced in February of this year. ProQR is one of the partners for the open access genetic testing program, demonstrating their patient-focused approach in supporting genetic testing. As a partner of the program, ProQR has access to expert physicians and de-identified data from IRD patients, which facilitate efforts to advance new treatments for IRDs.

Teaming with corporate partners to help promising therapies move through preclinical

and clinical development is central to our strategy. So we are very pleased to partner with ProQR. The fact that are there currently no available treatments for Usher Syndrome type 2A makes this work that much more exciting and critical.

We are pleased to have ProQR's founder and CEO, Daniel de Boer, on the call with us today to share update on their Usher, LCA and other IRD programs. Daniel is serial entrepreneur and passionate advocate for rare disease patients. He assembled a group of successful biotech executives as co-founders in 2012 and built team of more than 150 experienced scientists and drug developers, devoted to creating RNA therapies for patients in need.

Under Daniel's leadership ProQR has initiated clinical trials in multiple development programs for rare diseases and has raised over $300 million in funding, including completing an initial public offering in 2014. Without further ado, I would like to welcome Daniel and turn the call over to him. Daniel, please go ahead.

Daniel de Boer, ProQR Chief Executive Officer:

Thank you for the introduction and good afternoon, everyone. It is a great pleasure to join this April edition of the Foundation Fighting Blindness Insights Forum. I want to thank Ben, Jason, Chris and rest of the Foundation team for the invitation to speak here. It is always a great honor to be invited to speak to our key partners, particularly in this unprecedented time. I really hope everyone is staying safe and healthy.

I'm Daniel de Boer, CEO and founder of ProQR Therapeutics. I founded ProQR in 2012 when one of my own children was born and diagnosed with a rare genetic disease. I decided back then to see what I could do to accelerate development for rare diseases and started ProQR Therapeutics to do so.

ProQR Therapeutics is dedicated to developing life-changing RNA therapies for patients suffering from severe genetic disorders like Leber congenital amaurosis, Usher Syndrome and retinitis pigmentosa. We are based in the Netherlands in Europe and also in Cambridge, Massachusetts. Our mission is to help patients by creating RNA therapies that can stop vision loss or even reverse some of the symptoms of inherited retinal blindness.

I want to open my talk with outlining the cutting-edge science that sits at the heart of the medicines being developed at ProQR. In the state-of-the-art labs here in the Netherlands, our scientists are working day in and day out to discover and optimize ways to treat genetic rare diseases. We are specialized in the development of RNA therapies. These are medicines that consist of a short piece of synthetic RNA. This is

different from, for example, gene therapy or gene editing but it is the same outcome. We can take away the underlying cause of a genetic disease with this approach. In the case of inherited retinal disease, it would stop progression or even reverse some of the vision loss. RNA therapies are also what we call reversible, meaning we do not alter a person's genetic makeup permanently. We are aiming at creating safe and effective treatments to have a positive impact on people living with an IRD.

We are developing a pipeline of novel medicines for patients in need. Our pipeline focuses on diseases that are very severe and have limited treatment options available. All the medicines we are developing are RNA therapies and target the underlying cause in disease.

Just to give you a flavor of the work we are doing in clinical development:

First, we are conducting our Illuminate trial, Phase 2/3 study in patients over the age of eight years old who have Leber congenital amaurosis 10 or LCA10 and in particular for patients that have certain mutation CYS998X mutation in the CEP290 gene. The trial of sepofarsen began in April 2018 and this was a Phase I/2 trial conducted in patients. We published pretty encouraging good data from the trial in October 2019. The top line results that we showed were encouraging. The majority of participants showed meaningful improvements in visual acuity or functional vision after just three months and then maintained that improvement out through month 12. As an example, one participant who could only see light and dark at the start of the study could read letters on the standard eye chart after the treatment. Sepofarsen is now in a pivotal trial in North America, Brazil and Europe. If you want to learn more about this trial or see if you are eligible to participate, you can find more information at www.lcastudy.com.

Second, we are conducting our Stellar trial Phase I/2 study in adults with Usher type 2 or non-syndromic retinitis pigmentosa, a program in which we have a partnership with the Foundation Fighting Blindness. In a minute, I will tell you about the results of the first in human clinical trial that we are doing with QR-421a, which is the molecule we are studying here. This Phase I/2 clinical trial designed to evaluate the safety and tolerability of QR-421a and assessing treatment through the molecule. I want to strongly acknowledge the partnership with the Foundation Fighting Blindness here to help us develop QR-421a for Usher Syndrome. In the partnership as Ben mentioned before, the Foundation supports the trial financially and provides knowledge and know how on the disease, including through the learnings from the RUSH2A natural history study.

Thirdly, we are conducting our Aurora clinical study. This is a Phase I/2 study in patients

who have autosomal dominant retinitis pigmentosa, or adRP due to the P23H mutation in the RHO gene. We dosed our first patient in December 2019. In the study, we will dose up to 35 patients and we are conducting this study in several expert centers across North America. This trial is designed to explore the safety and tolerability of QR-1123.

Now moving on to the latest news. At the end of March, ProQR published findings of QR-421a. It is an investigational RNA therapy for the treatment of Usher Syndrome and non syndromic retinitis pigmentosa due to mutation of the USH2A gene. After all the participants in the first two groups had been in the study at least three months we did an interim analysis.

Findings from this analysis suggested QR-421a given as single intravitreal injection was safe. It showed early and encouraging evidence of activity, including improvement in sensitivity to light and an increase in ability to read letters on the eye chart. Two of eight participants in the two treated groups showed benefit in multiple outcomes measured in the treated eye. A similar response was not observed in the six participants in the control group that received a sham procedure. Based on these early positive findings we will continue the trial as designed.

The goal of the interim analysis in this 24-month Stellar trial of QR-421a was to assess safety and early signs of efficacy. We did this for the purpose of informing next steps in the development and future clinical trial strategy. We are pleased with the current safety profile and the early signals of activity thus far in the trial indicates that we are on the right path. The findings support continuing the trial as planned in order to identify a potential development path to registration.

Based on the safety profile and early evidence of efficacy observed to date, we are planning to expand to individuals who are homozygous for exon 13 mutations for the USH2A gene. In parallel, a high dose group is planned to start. Another interim analysis of safety and efficacy will be planned once all additional participants have reached at least three months of treatment.

If you are interested in participating in the QR-421a trial or any of the trials, please visit the ProQR website to find more information and contact information at www.proQR.com.

I also want to touch upon the COVID-19 situation we all find ourselves in. This has affected ProQR, which I'm sure doesn't come as a surprise to you. We made significant steps, in unprecedented times, to put the health and safety of our ProQRians, our trial partners, clinical trial participants and their caregivers first. I want to reassure you we are continuing to work relentlessly for people living with IRDs.

I would like to mention our partnership with My Retina Track program. Genetic testing is crucial to receiving an accurate diagnosis and to then move forward with the best potential care. The My Retina Tracker program supports the development of treatments and cures of inherited retinal diseases and we are delighted to expand our partnership with the Foundation. Please check out the initiative which can be found on the Foundation's website. If any of the above has piqued your interest, we have channels for the patient community specifically available on our website. You can find this on www.proQR.com. On the ProQR website, you will find the dedicated community page, the latest community updates and the ProQR Eye Connect newsletter. The ProQR Eye Connect newsletter is a specific update for you, the patient community, covering everything from our clinical development updates through charitable events.

I would also encourage you to also look at Twitter, FaceBook, and LinkedIn. There you will find daily and weekly updates on the work we are doing. Also worth noting, we just kicked off a new series called ProQR talks in which we will interview members of the healthcare community. I recommend you head to our ProQR YouTube page and check out the first episode with Usher advocates Molly Watt from the U.K. and Rebecca Alexander from New York – speaking about accessible information in an isolated time.

It is important for us to hear from you, the patient community, so please do follow us and ask us questions. Finally, I want to thank you for your participation and I want to thank our partners at the Foundation Fighting Blindness for this opportunity to participate in the Insights Forum.

Jason Menzo, Chief Operating Officer:

Thank you so much Daniel for the excellent update. On these Insight Forum calls, I must be honest, I learn something new every time even though I'm at the Foundation. This time I think the highlight that I will take away and remember is that the people who work at ProQR are officially known as ProQRians. That is awesome. I never heard that before. In all seriousness, the progress you guys at ProQR have made is really inspiring and we are thrilled to be partners with you and it’s exciting to consider the potential treatments for range of inherited retinal diseases you're working on. What we will do now is open the call to take questions and comments and if I could ask Chris to repeat the instructions for our attendees to ask questions.

Chris Adams, Vice President, Marketing & Communications:

Thanks, Jason. As reminder there are three methods for asking questions. First you may access the Q&A feature on the bottom of the Zoom control and type in your questions, which we have a few already there. Second, you can ask questions verbally.

To do so please select the hand raising function on the menu bar on the bottom of the interface and we will provide you with instructions to unmute yourselves. Third, if you joined by phone and not in the Zoom map, please submit your questions vie mail to info@FightingBlindness.org. Please note 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 in person over email over the next week.

Jason Menzo, Chief Operating Officer:

Thank you so much, Chris. Just as reminder as we are compiling questions, if you have questions for Daniel or from a ProQR perspective, Daniel has agreed to answer them but as CEO of a publicly traded company, there may be questions that are difficult or from a confidentiality perspective he is unable to answer, so keep that in mind there may be questions that arise that he is unable to answer based on confidentiality. It is about quarter till, so we will have about 20 minutes or so for questions.

I will package the first handful of questions that we have received all around genetic testing, so, Brian, I will address the first couple questions to you. First there are a number of folks asking how to enroll in My Retina Tracker and also how to get access to the free genetic testing program associated with My Retina Tracker. Let's start with how to enroll in My Retina Tracker and access genetic testing through it.

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

Yes, thank you, Jason. To enroll in My Retina Tracker is very straightforward. There is a website – you type into your browser myretinatracker.org. Single word, it will take you to the website. There you will be asked to sign up and register. You just navigate your way through and share a little bit of information about who you are, where you live, the disease that you have. By the way, none of that information about who you are, where you live or how we contacted you is ever shared with anyone outside the Registry staff. We always keep that confidential. When we talk about the de-identified data, which you will hear a lot about, that's what we mean. We will show the data but we will never show your name, your address, or how to contact you. Only staff of the Foundation will ever know how to do that. During that process you will be asked to read an informed consent. This will explain to you what the Registry is about, what data we collect, how we protect your privacy and it will ask you to check a box saying you agree. Once you have done that, then it will form a profile for you and once you got your profile or your accounts established, it will guide you through a series of survey questions to get your subjective view about your disease and how it affects your life and how you handle those aspects. The surveys are things that you can do in one

go if you would like to, or you can answer one or two and then stop and come back later on and answer them again. Those of you who are already in the Registry, I'm really excited to tell you that we are actually going to be launching a new software platform for the Registry. For those joining within the next few weeks you will see the old platform but shortly after that you will see a new platform. Don't be surprised if it looks a little different when you join. It is the same thing but a better, more stable platform for you. That's how you join the Registry.

For genetic testing, you do not need to be a member of the Registry. Obviously if you are a member of the Registry, it is really helpful. If you get tested and you choose to join the Registry after testing, please share that genetic data because that genetic data is really important in helping us to understand where specific forms of disease are in the community. To get genetic testing all you need to do is approach your eye care professional and if they are able to diagnose inherited retinal disease, then they are able to order the test on your behalf. There's a little bit of confusion that we have come across where some people think they can order the test themselves. That's not actually the case. You really need a clinician to order it. They order it from a very specialized lab.

Now, to get some information and guidance on that, you can go to the Foundation web page and look at the tab marked Genetic Testing and under there you find information not only about how to get genetic testing and how to join the Registry. On that genetic testing page, there are two documents you can download, one is for you – to explain the testing and what's involved for you, from your perspective. The other file is a document that you can download and take your eye care professional if they are not currently aware of the program. It gives them the information they need to go forward and order that test on your behalf.

Jason Menzo, Chief Operating Officer:

Thank you so much, Brian. There are a couple of additional questions building off of that.

Let me put them in front of you together as one question. If someone has already had a genetic test from their provider but not through the program, how do they put that information into My Retina Tracker. That's the first part of the question. The second is if someone has been previously tested and it did not identify a pathogenic gene mutation or identify the potential cause of their inherited retinal disease, will they need to be tested again as the science in genetic testing has advanced.

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

Yes, thank you. Two very good questions. Let me address the first one. If you have been tested and it didn't happen to be through our testing program, how do you get the data into My Retina Tracker. It is very straightforward. There are three ways it can be done. If your clinician would like to help you, you can ask them to go to the myretinatracker.org website. There's a tab there marked for clinician and the clinician can enter that information on your behalf. If they can't do that, you could do it yourself just by signing into your account and entering information or alternatively, you can send it to the staff at the Registry. I will give you email contact for that in a moment. We will enter that information for you and we will also attach the genetic testing report if you have a PDF or word document of the results. We can upload the report into your profile so it can be stored there. If you ever want to find it and you've misplaced your own copy, sign into My Retina Tracker and pull that down. The contact information is coordinator@fightingblindness.org. If you forget that, you can always reach us also through info@FightingBlindness.org.

The second question relates to the case of someone being tested and they did not find a mutation that could explain their disease. They are asking about next steps. Should they be tested and do they need to continue to be tested? This is really a fairly nuanced discussion that needs to happen with your clinician but the staff and Registry can help you initially. It really depends on what test you had in the past. People talk about genetic tests but there are many sorts of flavors of genetic tests. The flavor that the Foundation is currently running has almost 300 genes on the panel we test. We are testing 300 genes and these are nearly all of the genes that are known at this moment to cause some form of retinal disease.

Depending when you were tested earlier, you may have only had one or two genes or a few tens of genes tested. In that case, it is probably worth seeing if you qualify for the My Retina Tracker genetic testing and be tested on a full comprehensive panel.

On the other hand, if you have had a test within the last year or so and it's been a comprehensive test, then it is probably not worth your effort to be tested because the rate of discovery of new genes is not very great and those genes are hard to find and they normally only explain disease for a smaller group of people. You're better off to wait a few more years and do another comprehensive panel test. Now, that's fine, but how do you actually make that distinction? Well, if you go to the Foundation's website and look at the Genetic Testing tab, there you will find a patient download where you can download the information and see the exact criteria that we use to decide whether you do or don't qualify if you have had a panel test in the past. Again, if you're

uncertain about that, please reach out to your clinician or reach out to the Foundation staff and you can do that through coordinator@fightingblindness.org or through info@fightingblindness.org.

Jason Menzo, Chief Operating Officer:

That's great. Thank you, Brian. I have one more for you and then we will shift gears as I have a couple of them lined up for Daniel. The last question I will direct to you, Brian, is about information that's captured in My Retina Tracker and privacy. A number of the questions are along the lines of who has access to the information that's in My Retina Tracker, where does the information go, how is it kept private, and things along that nature.

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

That is a very good question and on the mind of many people. To start we take our responsibility with this data extremely carefully. Only a small number of people within the Foundation ever know who's in My Retina Tracker and that group is limited to four staff members who work with the Registry data. They are trained in human data protection, so human subject protection. They know the framework of the legal protection around that data and they adhere to it very tightly. So, for instance, people outside of that group of four are unable to know who is in the Registry. So, for instance, if Jason came to me today, we would not be able to tell him whether someone was in the Registry or not. We only tell the people who are in the Registry if they are in it or not.

We have a large number of protocols that we meet. There is a global data protection regulation that's standard within Europe. We are fully compliant with that standard. We are fully compliant with all U.S. standards and again, our view of privacy is that we never tell anyone who you are or how to contact you. If there's a clinical trial and someone has looked at your de-identified data, they may have seen you have a gene mutation and that your best corrected visual acuity is of a certain value and you have a specific clinical diagnosis, they may point to that profile and say we would like to get in touch with that person. The way it works is they would go through a formal process where they have an Institutional Review Board approve the reason they want to get in touch with you. That is then given to us. We review it. If we accept it, we then reach out to you as the person they are trying to contact and tell you they are trying to get in touch with you. We will share with you why they want to talk with you and how to reach out to them if you are interested in talking to them. The decision is totally in your hands. If you decide that you don't want to follow up on that particular opportunity or

contact being offered, you do nothing and no one knows. If however, you would like to follow up on it. You simply follow the information in the letter we send you and you connect with the third party who wants to talk to you. At this stage, it is up to you what you would choose to share or not and the Registry is no longer involved. That's between you and the other group. So again, we have many steps in place to ensure that when people look at the data they do not see anything that allows them to know anything below the state you live in. They certainly don't see contact information or your name or anything like that. Then when there is interest in you and that de-identified profile that you're sharing with industry and academic researchers, then it is in your hands whether you choose to reveal your identity or simply decline the offer.

Jason Menzo, Chief Operating Officer:

Excellent. Thank you so much, Brian. Let's shift gears and Daniel, I have a couple questions that have come in. I'm going to try to summarize them. But the first is around route of administration for RNA therapy. So you can speak to how the therapy is administered to the patient.

Daniel de Boer, ProQR Chief Executive Officer:

Happy to answer that question. RNA therapies for retinal diseases are administered through an intravitreal injection, that is a small injection in the side of the eye. The frequency of that injection is between once every six months and once every 12 months. So about once a year or twice a year, a treatment for RNA therapies in the eye. It is not a subretinal injection, it is intravitreal injection.

Jason Menzo, Chief Operating Officer:

I will try to summarize a bunch of questions that have come in around your platform and how it may be applicable to conditions and indications beyond what's currently being considered in your pipeline. Can you speak to other programs that are in your pipeline and speak generally to how the platform may be applicable to other indications than what you're currently studying.

Daniel de Boer, ProQR Chief Executive Officer:

We are developing these RNA therapies for inherited retinal diseases and we currently have three of these medicines in clinical trials and few dozen in preclinical stages of discovery and development. We are really expanding within inherited retinal diseases to see how far we can reach with this technology to help people with genetic retinal blindness. For example, we are looking into other mutations that cause Leber

congenital amaurosis and Usher Syndrome. The objective there is to use the same technology but just tweak it for other genetic mutations. Altogether, we think that our technology may be able to treat about 25 percent of the mutations that are known in genes in general and there is no reason to assume that that would be different for the eyes. We think with the technology, there's a significant opportunity to make a meaningful impact for the lives of people that live with retinal diseases. Over the coming years, I think we will unveil more and more of how we are developing the platform for other mutations and other retinal diseases and point people to our website, www.proQR.com, to see the latest on our pipeline and the latest developments as well.

Jason Menzo, Chief Operating Officer:

It is 2:00 here on the East Coast in the United States. There are a couple more questions that I think we can get to, so we will stick on for another couple minutes. This has been an amazing call from a participation standpoint and thank you to everyone who has submitted questions. We have received nearly 50 to 75 questions. So obviously we are not going to be able to get to all of them on the call. We really appreciate the engagement. What I will say the commitment has always been on these calls that if we haven't gotten to your question, then we will follow up with you personally via email. As we tend to typically get many of the questions that were asked about a particular individual's situation, such as, my child was diagnosed with X or I was diagnosed with Y and questions that are a little bit more medical advice type of things in nature. We tend to not be able to get into very specific guidance like that. However, I will assure you that anything that we can share on a personal one on one basis, we will do so individually after the call about specific questions or circumstances. Anyone who has questions about the diseases generally and what to do if newly diagnosed, there's a wealth of information on our website at info@FightingBlindness.org. We will respond to you individually with particular questions.

Shifting gears to COVID-19, there were a number of operational questions pivoting to expand around the statements I made at the beginning of the call. There have been a number of different specific initiatives that we have evaluated and that we are reducing cost associated with. We had the unfortunate news that we did reduce a couple of individuals from our team early on – about four weeks ago now. We are doing everything we can to do for there to be minimal impact on the service level and on our team and community broadly. And to work really hard to adjust expenses and shift how we are doing things to be as efficient as possible, which we always do anyway, but especially under these circumstances. Without getting more specific than that, I will say that we are doing the best we possibly can in very challenging situation from a

financial operational standpoint, with hopefully not any disruption in our service level to our community and certainly not to our programs and investments in the research that is our mission. The last question I'm going to pose to you, Brian, is just due to the fact there's so many questions specific to Stargardt disease. Could you give two or three minutes of a general 30,000 foot view statement around some of the current news related to Stargardt research at a high level and then we will wrap up the call with that.

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

Yes, I would be happy to, Jason. There's a lot of activity going on for Stargardt disease and the last time I looked at the clinical trials, there were ten clinical trials currently in progress for Stargardt disease. Many of them are at the Phase 1/Phase 2 stage trying to understand the safety of their therapy and how much of a dose they might need to give to see a signal of impact on the disease. But there are a couple that are later stage. For instance, there is a drug called Emixustat by Acucela, which is in a Phase 3 study, so that is quite an advanced stage. That's where they are looking to find definitive data that it could impact or change the progress of the disease. There is another drug, Zimura, by a company called Ophthotech, which is in Phase 2 and moving ahead. There is another Phase 2 study by a company called Alkeus.

All of them are showing some strong promise in treating Stargardt disease. Of course, we do have gene therapies and a variety of other small molecules being developed in that space. Behind those clinical trials, there are a large number of preclinical research programs which are progressing very well, many of which are in late stage development and which are looking promising enough that they will be heading to the clinic shortly. I think there's a lot of optimism with Stargardt disease and a lot of clinical activity going on.

Jason Menzo, Chief Operating Officer:

Thank you so much, Brian. And thank you to all of our panelists and participants. Brian, of course, Daniel and the team at ProQR, the ProQRians. And thank you, Chris and Ben, for participation and thank you everyone for today's call. Just to remind everyone, that there will be a transcript and audio recording of today's call, so that if there's information or pieces of details you didn't quite capture today that we will have that audio recording and transcript on the website within the next week. If you have any questions reach out to us at info@FightingBlindness.org. And there's a wealth of information at the website at www.fightingblindness.org. Thank you very much to everyone for participating. We will look forward to being with you and sharing great information again on our next Insights Forum quarterly call. Thank you all, stay safe and have a great rest of the day.