Foundation Fighting Blindness
***Insights Forum* Transcript**
March 20, 2024

Amanda Bement, Chapter Engagement Assistant:

Hello everyone, and thank you for joining today's Insights Forum. Currently all participant lines are muted and without video, please be aware that the controls are at the bottom of the Zoom window. This control bar may disappear when it is not in use. If you prefer to prevent the controls from hiding automatically, you can use the following keyboard shortcut to change the settings to always show meeting control. If you're using Windows commands, you can press the alt key, and if you're using a Mac keyboard, press command and backslash at the same time.

Today's presentation is being recorded, and is available with closed captioning. To activate the closed captioning, simply go to the toolbar at the bottom of the Zoom window, select Captions, and then Show Captions. Please note that on today's call, our speakers do have their videos live while they are presenting. However, all of the comments will be provided verbally, and there will be no slides. Throughout the call, you'll be able to ask questions via the Q&A feature, and the chat features, both of which are at the bottom of the Zoom window. We'll address these questions towards the end of the call. If we do not get to your question live, we will follow up over the next few weeks, so please make sure to include your name in your question. You can also submit a question by sending an email to info@fightingblindness.org. I would now like to turn the call over to our chief executive officer, Jason Menzo. Jason?

Jason Menzo, Chief Executive Officer:

All right, well thank you very much Amanda, and good afternoon everyone. Thank you for joining us today. We are very pleased to invite you to our quarterly Insights Forum webcast. As you know, we use these forums to provide updates on strategic initiatives here at the Foundation Fighting Blindness, and also to share research and development progress from across the broader landscape. As is

usually the case, we have a very full agenda for today's Forum. First, I'm going to get things started with a status update on our progress in developing our next five-year Science Strategic Plan. And then following that, I'm going to be introducing Mr. Jeff Klaas, who is our new Chief Strategy and Innovation Officer here at the Foundation, and he's going to share a little bit about his background, and perspectives on how we can strengthen community connections to support future plans and growth for the Foundation.

Then Chris Adams, our Vice President of Marketing and Communications, will provide an update on marketing initiatives, and ways that we're reaching out and engaging our communities. Next, Peter Ginsberg, our Chief Operating Officer, will highlight recent noteworthy corporate announcements and sponsorships, along with a summary of our fiscal year 2024 financial performance through the end of December. And then finally, Dr. Amy Laster, our Senior Vice President of Science Strategy and Awards will conclude our formal remarks with a summary of the latest high impact research grants that we have awarded. Then, like we always do, we will open it up for questions, and at that time we'll have several other members of the foundation's leadership team join us for the Q&A, including Dr. Todd Durham, who's our Senior Vice President of Clinical and Outcomes Research, and Dr. Rusty Kelley, Managing Director of our venture fund, the RD Fund. So, it is a packed schedule for today, and with that, let's go ahead and get things started.

It is hard to believe that it is already mid-March, and I've said on past Insights Forum webcasts, the pace at the Foundation and really in this field is nonstop, both on the research side, but then also as it relates to our team's execution of events across the country, and of course outreach to our community. But amidst these busy days, we are constantly thinking about, and focusing on what's next, and thinking about the future. And importantly, ways to advance our mission as the years ahead of us start to become a reality.

Much of the recent clinical and industry progress that our field has experienced in recent times has evolved out of basic and translational research that was previously funded by the Foundation Fighting Blindness. And in fact, 88% of the current clinically-staged programs in our field, and particularly in the largest areas of the inherited retinal disease field, 88% of those clinical programs came out of programs that were previously funded by the Foundation Fighting Blindness. And

the reason I bring that up today is that it really speaks to the importance and the power of our grants and awards programs to seed new innovations that in the future will hopefully evolve into future treatments and cures for these conditions.

And so now we are on the cusp of starting the next chapter in driving this type of research into the future. Every five years, we launch the next iteration of our scientific strategic plan, which identifies gaps that need to be addressed, and milestones that we can reach for, in the pursuit of furthering our mission. We are currently in the last year of our current five-year strategic plan, which spanned from fiscal year 2020 through 2024, and will conclude this June. We have been working with our Global Scientific Advisory Board to develop the next five-year strategic plan, and again, this will go into effect on July 1st, and extend all the way through 2029, if you can think about it that far into the future.

There are multiple steps currently underway in this process, and as background, our Global Scientific Advisory Board, which is made up of more than 60 esteemed researchers and clinicians from across the globe, has been reviewing achievements over the last five years, and identifying key knowledge gaps to inform the Foundation's research plans going forward. These insights are organized around our six research priority areas, which include novel medical therapies, gene therapy, regenerative medicine, cell and molecular mechanisms, clinical structure and function, and genetics. We also have a subcommittee specifically focused on age-related macular degeneration.

So, each of these priority areas has a program chair, and a committee of experts that are focused on that specific segment of our overall mission. At this stage, we are compiling the findings around the gaps and opportunities from each of these six areas, and it's being compiled into a consolidated document in coordination, and really being led by the Scientific Advisory Board chair for the Foundation, which is Dr. Jacque Duncan, who serves as chair not only of our scientific Advisory board. She's the chair of ophthalmology at the University of California, San Francisco.

We're working really closely with Dr. Duncan to develop a scientific manuscript which will be published in a peer review journal targeted for the research community, and there will also be a lay-friendly version of this gap analysis that

we will develop and draft specifically for the broader IRD community that will be available later this year on the Foundation's website. This gap analysis helps to provide the necessary context as our team sets the goals, and investment levels for the next five years, and we expect this new plan to be the most aggressive, and robust funding plan that has ever been put forth by the Foundation, and it will include specific strategies, and goals across our broad range of initiatives, including our grants and awards programs, our clinical studies, our genetic testing program, My Retina Tracker patient registry, and importantly the RD Fund, our venture fund, which as you know is making huge impact on our ability to advance our mission through investments into companies doing the work that are either near or in the clinic.

The culmination of this process will come together later this year, with the rollout of our new five-year Science Strategic Plan, and I look forward to sharing more details with all of you on a future Insights Forum call, as we progress towards the launching of this new plan.

Now, you may be wondering, how are we going to fund this new plan? It's great to have a plan, but you need the money to be able to fund it. And to that end, we determined that a near-term priority for us was to create a new role at the leadership team, at the executive leadership team here at the Foundation, focused specifically on developing new strategies, and innovations around our fundraising capabilities. And in November of this past year, I was thrilled to have Mr. Jeff Klaas join the Foundation as Chief Strategy and Innovation Officer, and you're going to learn in just a minute, he's a great guy. He brings a lot of talent and experience to our leadership team. He's a seasoned healthcare and technology non-for-profit leader, with an entrepreneurial spirit, and a commitment to leveraging technology and innovation to drive social impact, and revenue growth. Here at the Foundation, Jeff leads the departments of development, chapters, events and marketing and communications, and he has gotten up to speed very quickly, and is already making significant contributions and impact in positioning our organization for future success. I'm anxious for you all to meet Jeff and learn more about him. Now let me turn the call over to Jeff, so you can hear from him directly.

Jeff Klaas, Chief Strategy & Innovation Officer:

Thank you, Jason. It's exciting to be here on the Insights Forum today. I'm incredibly humbled, and grateful to be part of this team at the Foundation Fighting Blindness, helping to lead our organization in critical areas moving forward. I'd like to provide a brief perspective on the experiences that have prepared me for this unique role, and how my personal connection to the Foundation's mission makes it even more special. Then I'll share some key takeaways from my first 90 days on the job, and outline our key near-term focus areas.

I've spent most of my career working for community-focused organizations, emphasizing results-driven initiatives by connecting technology to outcomes. The model for the best-in-class globally impactful organizations is to get closer to the communities that they serve by harnessing the power of the volunteer. I'm eager to build on the strong relationships the Foundation has with community members. With a family member affected by a retinal dystrophy, I have witnessed firsthand the challenges and opportunities our community faces in dealing with blinding diseases.

Over the past 90 days, I've spent some time meeting with our chapter volunteers, board members, community members impacted by IRDs, donors, and fellow staff members, and this is truly an amazing organization. The team of staff and volunteers is strong. There are many opportunities to continue building on our community connections. That's the reason why I'm here.

Change is constant in our dynamic world, and as an organization we are no strangers to it. In fact, our ability to adapt, and evolve has been a key factor in the success of the Foundation.

We recently announced a transformation in the way we connect with our global network. Our aim is to create a structure that better aligns the Foundation's staff with our chapters and communities, fostering increased local engagement and relationships, and we believe that this change will enhance our ability to make a more significant impact on our mission. So, through this realignment, a new structure has been deployed, focusing on further strengthening our existing chapters, and expanding in the new markets.

The structure is chapter-centric, rolling up into defined territories and regions. Our regional directors and field staff are focused on recruiting and collaborating with volunteers, raising awareness, increasing corporate partnerships, and implementing fundraising strategies, and mission-related events to achieve our revenue goals. Together, we're not only refining our structure, but amplifying our impact. This will empower our team to cultivate stronger relationships with you, our valued global community members, forge strategic partnerships, and explore novel avenues for fundraising, and resource mobilization around the world. We are creating a more agile, scalable, responsive structure that aligns seamlessly with our organizational goals. We envision the realignment of the development team to drive revenue through all channels, increasing Foundation-funded research, which is critical, because science is outpacing the funding.

We're moving closer towards you in the coming months, in the coming years, as we activate and become a truly global leader, we're going to need your help, and it's not just fundraising. We need volunteers and leaders to advance our mission. There are opportunities to serve as chapter volunteer leaders, chairs of committees, community events, and local early detection and prevention programs. Get involved today by visiting our website at at fightingblindness.org/volunteer.

If I could suggest one takeaway for you today, it is to get involved. You are participating in this forum today, so we already have a shared purpose. We want all of you around the world to be empowered to use your knowledge and resources to share it with your families and community. We can harness the power of collective energy to fund the research that will provide cures and treatments today, and for generations to come. I look forward to sharing this journey with you, and my colleagues here at the Foundation. And now, I'm pleased to turn over the call to our Vice President of Marketing and Communications, Chris Adams.

Chris Adams, Vice President, Marketing & Communications:

Thank you Jeff, and good afternoon everyone. Our team is focused on increasing outreach, support, and engagement within our community and beyond. On the call today, I will highlight several of these initiatives and upcoming events.

February was Age-Related Macular Degeneration and Low Vision Awareness Month. The goal of our month-long campaign was to raise awareness, and garner community engagement by providing educational content for our followers to share. We partnered with several content creators to develop a video series called Low Vision Tool of the Week. Each week we highlighted a new tool, or feature including the ReBokeh App, Sam Seavey's The Blind Life, Oko App, and Ocutech Bioptics.

To honor International Day of Women and Girls in Science, Dr. Esther Biswas-Fiss, a professor and chair of the Department of Medical and Molecular Sciences at the University of Delaware, was featured in a recent Inspiring Beacon story video sponsored by Johnson & Johnson. Last year, Dr. Biswas-Fiss received one of the Foundation's Individual Investigator Research Awards for her work in deciphering the impact of ABCA4 Genetic Variants in inherited retinal disease prognosis. Definitely check out this Beacon story, and many others in the Stories & Resources section of our website.

We also hosted our second annual 29 Mile Walk Run Facebook challenge in which community members walked or ran 29 miles in the month of February to raise awareness for the Foundation. And speaking of walking, this spring, we will host 20 Vision Walks in communities across the country. These fun, family-friendly events bring together hundreds of teams, and thousands of walkers as we step towards treatments and cures for blinding retinal diseases.

We kicked off our spring event season with our walk in Phoenix, Arizona on Saturday, February 24th. The event raised more than $100,000, and attracted more than 500 walkers. To learn more about our Vision Walk program visit Visionwalk.org.

In addition to the walks, we also have many special fundraising events throughout the year taking place across the country. These events, which are led by our many dedicated volunteers, provide a meaningful source of funds and community engagement. For example, the St. Louis Night for Sight Gala will be held on April 25th at Grant's Farm, and our annual New York City Night for Sight Gala will take place on May 9th at the Lighthouse at Chelsea Piers in Manhattan, the gala celebrates beacons of the fashion, food, and finance industries, as well as the

blindness community. At this year's event, the prestigious Visionary Award will go to Stephen Rappaport, CEO of L'Amy America, and Noah Katz, President and CEO of PSK Supermarkets. Tickets and sponsorship opportunities are still available. You can learn more about all the upcoming events by visiting the Foundation's website at www.fightingblindness.org, and navigating to the Foundation Events tab at the top of the home page.

Our marquee event this year is Visions 2024, the national conference of the Foundation Fighting Blindness, which will be held on June 22nd to the 23rd at the Marriott Magnificent Mile in Chicago. The conference provides an excellent opportunity to connect with others from the blind and low vision community, and learn about the latest research advancements, products, and services for members of our community. And we are so excited to share that our keynote speaker will be Sumaira Latif, accessibility leader at Procter & Gamble. Sumaira, who also goes by Sam, is P&G's first company accessibility leader. She and her team are responsible for driving forward innovation to make the company's more than 65 brands inclusive, and accessible for people around the globe who experience some form of disability.

Sam was born with retinitis pigmentosa, and by the age of 16, she lost 97% of her sight. Sam will share her inspiring story of overcoming blindness, dealing with rejection, forging a successful career, and working to make a world that is more inclusive for all.

This conference is going to be packed full of outstanding sessions, including Sam's keynote speech, and there will be many opportunities to network. You can view the schedule events, the special speakers, register, and reserve a hotel room by visiting our website, fightingblindness.org, and clicking on the Visions 2024 tab on the top right of the home page. We encourage you to sign up now, and join us in Chicago in June.

I'm now pleased to turn the program over to Peter Ginsberg, our Chief Operating Officer. Peter?

Peter Ginsberg, Chief Operating Officer:

Thank you Chris. I'd like to begin our funding and financial summary today by recognizing our corporate sponsors providing key support for important foundation initiatives. We're excited to announce a new relationship with Amgen, the largest biotech company in the world, Amgen markets the thyroid eye disease drug, TEPEZZA, and Amgen really values the breadth of our network of nearly 50 volunteer-led chapters, and it's providing broad support as a Vision Walks and outreach sponsor. We're very grateful for Amgen's commitment and involvement in supporting the Foundation, and we look forward to a very long and fruitful relationship with Amgen.

We also have tremendous corporate support for events that the Foundation hosts throughout the year. We'd like to recognize Atsena Therapeutics and Johnson & Johnson for their lead sponsorship of our annual Investing in Cures Summit, which the Foundation and RD Fund hosted earlier this month. This event featured presentations by the world's leading innovators in our field, and was a fantastic event.

In addition, we have a strong lineup of corporate sponsors supporting our upcoming Retinal Cell and Gene Therapy Innovation Summit, and those sponsors were led by J&J and Spark. We're continuing to add sponsors, and we welcome outreach from interested organizations. This Innovation Summit, which is co-hosted by the Foundation and the Casey Eye Institute of Oregon Health and Science University on May 3 in Seattle, occurs just before the major annual ARVO meeting. And at our Summit, representatives from biotech and pharma industries come together with members of the clinical, and scientific communities to discuss rapidly emerging therapies, and strategize how to move the most advanced retinal disease therapy options forward. And that event is coming up in May, but I'm told it's already sold out. This is just a great event that brings the top research together in one place, in early May.

As Chris mentioned, our major community member focused event is Visions 2024, and that will take place in June, with a packed agenda of educational, inspirational and networking sessions. This event is made possible through the valuable support of participants and sponsors, including lead sponsors, J&J and Alkeus. I

also want to note a number of other companies sponsoring these Foundation events including Annexon, Apellis, MeiraGTx, Nacuity, Ocuphire, Restore Vision, Rhythm Pharmaceuticals, SparingVision, Two Blind Brothers, ReBokeh and Feeldom. So we really appreciate their support.

I'd like to next provide our quarterly financial update. The Foundation operates on a fiscal year that runs from July to June, so we've now passed halfway through our fiscal year 2024. And for the first six months of the year through December 31st, our unrestricted fundraising revenue was $16.2 million against the operating expenses of $9.0 million for a net fundraising surplus of $7.2 million, and that places us on track for our fiscal 2024 budget in which we're targeting $13.4 million in net fundraising surplus to support the new research funding that's so important to our community.

I'd like to conclude my remarks today by highlighting several noteworthy recent corporate developments moving forward new therapeutics for these blinding retinal diseases.

First, Laboratoire Thea, which is the leading European developer of eye care products, completed the acquisition from ProQR Therapeutics of two emerging antisense treatments for inherited retinal diseases. These emerging therapies for LCA10 and USH2A provide hope for preserving and restoring vision for patients. And the RD Fund, which is our venture philanthropy arm invested in the development of the USH2A program, and supported ProQR’s effort to find a buyer for both of its ophthalmic assets. We look forward to future updates from Théa as it continues clinical development of these two important programs.

Also, Ascidian Therapeutics received authorization from the FDA to launch a Phase 1/2 clinical trial for its RNA editing therapy for people with Stargardt disease, which is an inherited form of macular degeneration caused by mutations in the ABCA4 gene. Ascidian plans to begin enrollment for the clinical trial this year.

Also, Beacon Therapeutics reported positive data from its Phase 2 SKYLINE clinical trial of its X-linked RP gene therapy. This emerging gene therapy is for patients with mutations in RPGR, which is the gene most frequently associated with XLRP.

Foundation Finding Blindness funded successful canine studies of this XLRP gene therapy at the University of Pennsylvania School of Veterinary Medicine that help make this XLRP gene therapy clinical trial possible. And importantly, Beacon plans to launch its Phase 2/3 clinical trial for this gene therapy in the first half of 2024, so coming very soon.

Our track record in funding highly impactful research every year comes from the dedicated work of our staff and volunteers, combined with the generous support of our donors, sponsors, and foundation partners, which we greatly appreciate. I'll now turn the call over to Dr. Amy Laster to provide a research and science update. Amy?

Dr. Amy Laster, Senior Vice President of Science Strategy and Awards:

Thank you so much, Peter. Good day everyone. On today's call, I'm going to provide an update on our current research funding, summarize some new grants that have recently been awarded, and highlight several recent scientific and medical industry events. The Foundation is able to fund a very diverse portfolio of research. This includes basic science and emerging therapies to address the entire spectrum of inherited retinal diseases, and dry AMD, for all patients affected, no matter what specific gene mutation is causing their disease, or the severity of their vision loss.

We're currently funding 93 active grants, which are conducted by 96 researchers at 71 institutions, eye hospitals and universities across the world.

Based on a rigorous review by our executive scientific advisory board, we awarded three new Clinical Research Fellowship Awards for 2024. These include Dr. Kirk Stephenson at Hospital for Sick Children in Toronto, who will study the natural history of vision change in PRPF31 related autosomal dominant RP patients.

Dr. Kirill Zaslavsky at Massachusetts Eye and Ear will use genetic data and health records from volunteer biobanks to better understand the link between genotype and phenotype in dominant pathogenic variants that's associated with inherited retinal diseases.

And third Dr. Ahmad Al-Moujahed also at Massachusetts Eye and Ear will investigate the genetics of cystoid macular edema in RP patients, and this is to determine whether there's a relationship between that condition and different genetic causes of RP. And if so, what's the impact on response to treatment for cystoid macular edema.

In addition to these, we recently announced a new, enhanced Career Development Award, which is given to mid-career clinician scientists. The recipient is Dr. Ajoy Vincent from Hospital for Sick Children in Toronto. He is leveraging a gene discovery from an earlier Foundation award that resulted in the creation of a new mouse model for a hereditary macular dystrophy. So, he's going to follow disease progression in this model to really understand the disease mechanism, and then to follow a treatment approach using different dietary supplements to improve retinal health and prevent, or slow disease progression.

These grants are only the first of many awards the foundation will make this year. We will soon announce the recipients for our inaugural PRPH2 and Associated Retinal Diseases Research Science Award program, the Free Family AMD Award, and our Translational Research Acceleration Program. The translational awards are targeted to accelerate movement of particularly promising pre-clinical research towards the clinic. We have five other award categories, which are at varying stages of the application and review process. These include our Career Development Award, the Individual Investigator Research Award, the Clinical Innovation Award, the Program Project Award, and finally, the Non-Rodent Large Animal Award. These we anticipate announcing early summer.

We are honored to help fund the promising research that's taking place globally, and we're very grateful for your support that makes this possible.

In addition to funding research, the Foundation takes a leadership role in conducting collaborative events that will help move our field forward. There were two recent prime examples of community events sponsored by the Foundation. First, earlier this month, we convened a virtually externally led patient-focused drug development meeting on dry AMD. This meeting enabled patients living with dry AMD and caregivers to share their personal journey in a live discussion with drug developers, researchers, regulators, as well as healthcare policymakers and

payers. The takeaways from this type of meeting are to inform clinical trial design, and benefit risk decision-making for evaluating and approving treatments for dry AMD. This event was made possible through the support of Apellis, Astellas, and Novartis as presenting sponsors.

Last month, the The Stargardt's Summit was hosted by the Foundation Fighting Blindness, along with Prevent Blindness, the Carroll Center for the Blind, and The Blind Life. This virtual summit enabled participants to learn about the newest Stargardt's research, explore adaptive living resources, hear impactful patient stories, and connect with others going through similar journeys. Both of these events are now available for replay on the Foundation website, so I encourage you to check them out. With that, I will now hand the program back to Jason.

Jason Menzo, Chief Executive Officer:

Well, thank you so much, Amy. And before we transition into the Q&A session, which we'll start here in just a moment, I do want to highlight one additional very important recent development. And that is earlier this month, a bipartisan bill was introduced into the US House of Representatives by Representative Brian Fitzpatrick from Pennsylvania, and Representative Sanford Bishop from Georgia. The bill, which is titled The Loans for Biomedical Research Act, would authorize a limited federal guarantee for low-interest, long-term loans to pharmaceutical and biotech companies, and developers in academia. These loans would then be packaged into an investment instrument known as a BioBonds, and the term BioBond is actually something that we refer to this act as pretty routinely. So, if you get emails from the Foundation, or if you're interested in learning more, you can go to biobonds.org, but these BioBonds would be sold on the open market to long-term investors, such as insurance companies and pension funds.

The legislation that I'm referring to today would provide up to $10 billion a year for three years in guarantees for bonds comprised of eligible loans. And it's important to note that while we believe this bill can have profound impact in our space, and on our mission, that it's not exclusive to vision research, it really would be available to programs across the entire biomedical landscape, from cancer to diabetes, and of course to blinding diseases. And I really do encourage you to learn more at biobonds.org. And of course, if you're comfortable doing so, reach

out to your representative, or to your senator, to ask for the support in this important piece of legislation. Of course, if you'd like to learn more about BioBonds, or if you'd like to learn more about the Foundation Fighting Blindness, learn more about the research pipeline, the clinical trial pipeline, all of these resources, and much, much more are available at our website, fightingblindess.org. And of course, at any time if you have a question for the Foundation, for any of us, you can always email us directly at info@fightingblindness.org.

And one final comment as I wrap up our formal remarks today, I do have one additional piece of information I'd like to share, and that is that earlier this month, Dr. Claire Gelfman decided to step down as the Chief Scientific Officer here at the Foundation, and has transitioned into a new role supporting me specifically as a senior scientific advisor. I just want to take a moment to thank Claire, and share with you all that during her tenure, her enthusiasm and passion for our mission really did help propel many of our initiatives forward. I'm grateful for her dedication, and really, especially for the relationships that she's cultivated with so many of you, the members of our community.

I have begun the process of identifying a successor and we'll share progress on that as appropriate with you on these calls. And in the interim, I'm working really closely with our very, very strong science team, who will continue the valuable work towards our mission.

And now with that, we are ready to shift and open up the call to questions, which is terrific, because it's 1:35 here on the East Coast, and usually we're up against the clock. So, we've got 25 minutes today for questions, which is terrific, and we've already had a dozen or so questions already chatted in, and we'll get to as many of them as we can. Amanda, let me ask you to please reiterate how to ask questions for our guests here today.

Amanda Bement, Chapter Engagement Assistant:

Thanks, Jason. There are several methods that you can use to ask questions. You can submit them through the Q&A or chat function, both of which are at the bottom of your zoom screen. And please do make sure that you include your

name, so that we can follow up afterwards. You can also send an email to info@fightingblindness.org, and we will follow up in the next week.

Jason Menzo, Chief Executive Officer:

Very good. Well, thank you. Can I invite my colleagues, Chris, Amy, Jeff, Rusty, Peter, Todd, to please come off mute. Please turn your cameras on, and we'll go ahead and start with the Q&A session. I'm going to actually address the first question to you, Rusty. So, there's a question that was chatted in, and this individual, thank you, whoever you are, but chatted in four or five questions, which were all very good questions. The first of which I'm going to address to you, Rusty, is about the NACA trial, and that's specifically referencing Nacuity, which is a company in the RD Fund portfolio of course, but then also what we know perhaps about NAC, the NAC Attack trial that's also fielded here at the same time, their specific question is its use either, of these programs, in pediatrics. And so let me turn it over to you to answer that question.

Dr. Rusty Kelley, Managing Director of the Retinal Degeneration Fund

Thank you, Jason. Thanks for the individual that posted the question on NAC, and NACA. This is a short answer. Neither trial has or is enrolling pediatrics. This is for patients that are 18 years and older. The NACA trial, which is N-acetylcysteine amide within the company, Nacuity, in the RD fund portfolio is fully enrolled. That was a phase two trial in Australia, and that data is in a follow-on state, and there's interim analysis being conducted momentarily.

Jason Menzo, Chief Executive Officer:

Very good. Thank you, Rusty. And there's a number of questions I'm going to come to you with, Rusty, over the course of the next few minutes, but let me go to Amy next, and Amy, let me ask you about... I think this is a little bit dated, but there was a period of time, maybe 10 or so years ago, where there were some questions around the use of hyperbaric oxygen therapy chambers to stop or slow RP. Maybe you can speak to that, Amy.

Dr. Amy Laster, Senior Vice President of Science Strategy and Awards:

Thank you. There was a study, again, as you said, it was about 15 years ago, where researchers tried to assess the role and usefulness of this particular therapy on rescuing photoreceptors, or preserving vision. However, the evidence regarding the impact of this therapy in RP, it's really still conflicting and there haven't been additional studies to really understand the effectiveness in real world clinical practice. So, what I will say is consult with your healthcare professional for any personalized medical advice. It's really important to note that while this particular study provided some insight, it's not conclusive to prove that this particular therapy is appropriate for RP, and everyone's individual health situation is really unique. And so, even what works for one person may not work for another.

Jason Menzo, Chief Executive Officer:

Thank you, Amy. I'm going to come to you, Peter Ginsberg next, which it's kind of interesting because this is a science related question, and you are Chief Operating Officer, but in many ways you wear so many hats. Peter, you've been really involved in our interactions with a new agency within the federal government called ARPA-H. And this question is around any studies or trials on the advancements regarding either retinal transplantation, or perhaps even eye transplant. And of course, we know that that is the primary focus of a program within ARPA-H called the Whole Eye Transplant. So Peter, maybe you could just briefly speak to the program and ARPA-H as a whole.

Peter Ginsberg, Chief Operating Officer:

Sure, Jason. And the background here is that the Foundation has long been focused on whole eye transplant as a potential solution to improve, or restore vision in individuals affected by retinal diseases. There is a new research funding agency, as Jason noted, called ARPA-H. ARPA-H stands for Advanced Research Projects Agency for Health. ARPA-H is funding a very large new program focused on the development of whole eye transplantation to ultimately restore vision in affected individuals.

The technologies in this arena are still fairly early stage, but with the ARPA-H program, the goal is to bring new opportunities through whole eye transplant to

the community in six years. It's a very aggressive timeline, but the plan is to have a number of programs developed that might ultimately benefit patients within six years. So, the Foundation was excited to see ARPA-H having a program in this important arena, and we've built a large team of world leaders in the whole eye transplant research world to submit a proposal to ARPA-H that would play a critical role in the effort to develop whole eye transplant. We're really excited about this opportunity, and hope to have additional information for you in the coming months.

Jason Menzo, Chief Executive Officer:

Great, thank you, Peter. Similar, there's been some recent advancements in... It's somewhat similar, I guess if you think of whole eye transplant as one strategic way to address not just conditions within inherited retinal disease, but really blinding conditions due to a variety of different causes. There's been some technology, and some advancements in an adjacent field, which we call prosthetics. And Rusty, I know this is something that you've begun looking at, or we've begun looking at. It's a fast moving field. There's quite a bit of history. There's an approved product that was in the market around this several years ago, but maybe you could share just the current state of play and what we know about prosthetics, and how it's fitting into our plans in the next couple of years, Rusty.

Dr. Rusty Kelley, Managing Director of the Retinal Degeneration Fund

I'll start with the strategy of fitting in more potential devices, including prosthetics, therapeutic devices that is, into the FFB portfolio, as well as those that are investable in the RD Fund portfolio. There's a number of different classes of prosthetics that we've learned recently. The RD Fund has yet to invest in one of these devices, but we're certainly studying them with our advisory board. There's some that are non-invasive, there's some that are more invasive, but generally the goal is to simulate and or magnify vision as opposed to some of the more invasive renewal strategies like optogenetics or cell-based therapies. These are usually external devices that provide electrical currents into the eye that can stimulate activity and metabolism. But as Jason indicated, we're at the early

stages of trying to better understand some of these devices, but there are a few that we believe are potentially investable. More to come as we learn more.

Jason Menzo, Chief Executive Officer:

Okay. Thank you, Rusty. I want to shift now. We talk a lot about the different types of strategies, and the different types of interventions that we're pursuing. You all will hear us say terms like, "gene agnostic," or, "gene specific," or we talk a lot about My Retina Tracker Registry, which I'm going to shift in a second here and turn it over to Todd to talk a little bit about My Retina Tracker. And there's a number of questions. I know this is really important to our community, because there's a number of questions that are about these topics.

Like, "What if I've been genetically tested in the past, but a pathogenic gene or a known mutation was not identified? What now?" or, "How do I get genetically tested to understand if it is possible to identify the gene or mutation that's causing my condition? What is happening with gene agnostic approaches?" This is where I want to shift the discussion and maybe Todd, I'll turn it over to you now just to talk a little bit about the My Retina Tracker Registry, how one would get tested, and ultimately the elusive gene aspect to what happens when the report comes back and there isn't a pathogenic gene, or mutation identified.

Dr. Todd Durham, Senior Vice President of Clinical and Outcomes Research

Thanks, Jason. My Retina Tracker Registry is a program that we sponsor here at the Foundation Fighting Blindness. This is a way for us to capture the experience of individuals who've been diagnosed with inherited retinal diseases. And since 2017, this program has also included a sponsored genetic testing program that has been available to individuals in the United States whose healthcare providers have diagnosed them with an inherited retinal disease, and they suggest, or prescribe, for their patient a genetic confirmation of that diagnosis, and that test is available through this program to patients at no charge.

So, this program has provided a great deal of impact to individual patients, and their providers who are looking to take good care of their patients, but it also gives us great research value. When the reports come back from this program and other clinical panels. What we've found and others have reported is that in about

60% of cases, individuals with inherited retinal disease will get a clear cut definitive diagnosis from this test, from this panel test, but that leaves off a significant portion of individuals who don't have a clear cut answer as to what's causing their inherited retinal disease. And the reason why that can be is for a number of reasons.

In some cases, that report is not conclusive because maybe we don't have enough evidence in your report. There are variations in your DNA that haven't been identified with enough cases. So, those would be called variants of uncertain significance, and that the idea there as we think that this genetic mutation is causing your disease, but we don't have enough evidence to say for sure. So, that's one cause. Another is also of interest is some genetic changes that occur in the non-coding regions. Those are called introns, can also be attributed to your disease, and those are harder to find, and they're not typically picked up on our commercially available panels. And another reason why your result might come back is inconclusive or null is there might be a genetic change elsewhere that's not even in the code itself, but maybe a structural variation, also more difficult to detect using our commercially available testing methodologies.

So, the main point here is there can be a number of reasons why your result is not clear cut, but importantly, the Foundation has funded in the past, and continues to fund as a priority, the search for unknown cases of inherited retinal disease through our research program. And Jason, if I may just take a minute, someone wrote in to us on the chat box that he or she participated in the ProgStar study, and I just want to say to him or her, "Thank you very much for participating in the ProgStar study." Here at the Foundation, the studies that we sponsor and fund, we share our data with researchers in perpetuity. It's available for researchers. And in this particular case, the ProgStar study data have been accessed by dozens and dozens of companies who are looking to treat ProgStar. So, thank you for being an innovator and a pioneer.

Jason Menzo, Chief Executive Officer:

Right. Thank you. Thank you, Todd. That was a terrific shout-out, and we appreciate the individual for participating, so thank you for that. Let's shift now and talk about different types of intervention strategies, or different types of

potential treatments. And again, Todd, you just spoke of the Registry, and you talk about genetic testing, which if a gene is identified, there are plenty of strategies which we'll talk about later in this call too, that are gene specific, but importantly, there are plenty of folks, as Todd just referenced, that a gene or mutation is not identified.

And so, there needs to be strategies to bring potential treatments forward that are what we call gene agnostic, or independent of the gene that is causing the condition. And there's different strategies even within the gene agnostic segment of potential treatments. And so Amy, maybe I could ask you to speak to just gene agnostic as a whole, but then specifically when we talk about optogenetics, or cell therapy, what is happening in that segment? And then after Amy, Rusty, why don't you go right from that to talk about a couple of the strategies that are within the RD Fund portfolio?

Dr. Amy Laster, Senior Vice President of Science Strategy and Awards:

Thank you. With the gene agnostic, essentially what researchers are attempting to do is to solve the cause of disease without having to specifically target a gene. So, perhaps you might have a gene, and it'd have lots of different mutations in it, in different places across the gene. Well, if you're only targeting one mutation, then that therapy will work for that mutation within that gene, but maybe not for the other mutations. Gene agnostic is to resolve the disease without having to specifically look at a mutation. And as Jason already alluded to, there are different levels of gene agnostic approaches. One that is being developed and is typical for what we call late stages of disease when most photoreceptors are gone, or non-functional, are optogenetic approaches, as well as cell-based therapies.

So, with optogenetics, this is a therapy where the cells that are typically light sensing, our photoreceptors, they're gone or they're not functional. And so researchers have developed ways to take proteins, or chemical molecules that can detect light, and insert those into cells within the retina that usually don't, but now they can, because they're still attached to the pathway that goes to our brain, and help us to interpret light and vision as we know it. So, that's one approach, that we call gene agnostic for late stages of disease.

The other is cell-based therapy. And so, this is really taking photoreceptor cells, so these cells can be generated from the patients by doing IPSC cells, or induced pluripotent stem cells, or precursor stem cells, or sometimes researchers take retinal cells, and are working to convert them to photoreceptors. And so, either transplanting these cells into the space of the retina, where those photoreceptors have degenerated, or as I said, taking cells that are existing there, and then converting them to photoreceptors. And so, in both of these approaches, again, these are approaches that are really for individuals who have lost all of their photoreceptors, or function. Rusty?

Dr. Rusty Kelley, Managing Director of the Retinal Degeneration Fund:

I think the natural transition from Amy's comments into the RD Fund portfolio are which of those gene agnostic approaches exist within the portfolio? And it's always great to start with Sparing Vision, the first company that the Foundation, the RD Fund invested in, and its two lead programs are both gene agnostic. One is called rod-derived cone viability factor, and this is a factor that's produced by the rods among the photoreceptors that protect the cones. This is being delivered by an AAV-based gene therapy strategy, and it more broadly applies to patients with intermediate disease, irrespective of their gene variants. They also have a follow-on program that, as Amy described, is classified as an optogenetic strategy that's referred to as GerC1, and it also targets cones in intermediate to later stage disease. But certainly the cones have to be present, so not quite fully advanced disease.

We also have Nacuity, we talked about Nacuity earlier, and the N-acetylcysteine amide compound, NACA, that's being evaluated in a Phase 2 Australian trial. That's also gene agnostic, and it's an antioxidant that has been shown to protect the retina from degenerating further, also irrespective of the gene cause of the disease. That's being first evaluated in Usher disease related to RP. And the goal there is to expand the reach of indications beyond Usher. I think we speak about IRDs heavily, and that's sort of the basis of the Foundation, but we also cover dry AMD, and we do have a gene agnostic approach as well, and that's in a company called NVasc, founded by the great Napoleone Ferrara, who discovered VEGF, and

subsequently developed those VEGF therapies for wet AMD, but this is a vascular mechanism that's being applied to dry AMD and it's also gene agnostic.

Jason Menzo, Chief Executive Officer:

Very good. Thank you, Rusty. We've got about five minutes left and as is always the case, I don't know, 20 questions that are in the queue, so we're not going to be able to get to all of them. I do assure everyone who chatted a question in that our team will go through, and we do every time, and identify all the questions and we'll reach back out to you individually to answer them to the best of our ability. As a general rule, I do want to clarify that the Foundation Fighting Blindness, of course, is a global leader in driving the research to advance treatments and cures, but what we are not are doctors in the sense of medical doctors, clinicians. And so, any medical advice things like, "What should I do about," or, "How should I treat X," Or, "If you were me, what would you do?" It's really not where we play, and we will always refer you to a medical professional, an ophthalmologist, particularly a retinal specialist, or an optometrist that specializes in inherited retinal disease.

We have resources to help introduce you to appropriate clinicians. So, if you have a question or a need to get in touch with an expert, don't hesitate to ask us. But sometimes there are questions that we get that we're really not equipped to answer because we don't give medical advice. We drive the research to provide interventions, treatments and cures. So, with that little disclaimer, let's shift to the next two questions I'm going to ask you at the same time just to save time, Amy. One is there was a recent publication a week or two ago around the role of gut bacteria, and its potential impact in CRB1. That was question one. And then question two, for many, many years, and there was actually research funded by the Foundation, there was a belief and sort of a standard that vitamin A was routine standard of care for individuals with certain types of inherited retinal diseases. And then recently there's sort of a 180 shift on that, and that there's new news around that. And so, maybe you could speak to both of those topics.

Dr. Amy Laster, Senior Vice President of Science Strategy and Awards:

Sure. Thank you, Jason. In fact, both of these came out of recent scientific publications that have been circulated in the community, and the first one I'll talk about is the vitamin A. That publication came out last summer. And so, this was a follow-up study, as Jason mentioned, to a Foundation funded study that was done at Mass Eye and Ear to evaluate the benefit of vitamin A for RP. And in the study, they used the same patients, taking their blood samples as from the original study, but they've also added some additional data. And so, they also wanted to see if certain genetic subgroups of RP responded to a vitamin A regimen. But the sample sizes for these different genes, they really weren't large enough to kind of draw any conclusions about subpopulations.

But the outcome was that vitamin A, it doesn't provide any benefit for vision. And they also looked at vitamin E supplementations and they found that to be deleterious for vision. As Jason said, and I'll sound like a broken record on this, you should always consult with your doctor about taking vitamin A, or any supplement. We did hear from one of the study clinicians that he's okay with people continuing the regimen if they feel that they're doing well on it, and for him and his patients, he will continue to monitor those patients if they choose to continue taking it. So, please talk to your healthcare provider with regards to any vitamin supplement.

On the other research paper that came out more recently on CRB1 and gut bacteria, we did seek input from our Scientific Advisory Board, and they noted that there are some issues that might affect the conclusion that the paper drew. It talks about a condition in mice where the gut, which is usually a really tight barrier, becomes leaky and allows things to pass through that normally wouldn't. So, this along with changes in the gut's bacteria, which is what we call the microbiome, might have some effect on how the disease progressed in mice, but the experts believe that this isn't the main reason that CRB1 disease happens.

So we don't really have any evidence to suggest that what happens in mice would also happen in the human. So, it's really, really uncertain if these findings apply to people. And specifically patients with CRB1 disease, there haven't been any kind of reports of leaky gut, but this is going to be explored further, or I should say, needs to be explored further. So, while the paper provides some really interesting insights, it's very important to kind of take the findings with a grain of salt. And at

this moment, we can't draw any definitive conclusions without further research. And again, I'll end, please consult with your healthcare professional for medical advice. Thanks, Jason.

Jason Menzo, Chief Executive Officer:

Thank you, Amy. And I will echo that point, and also offer a resource that the Foundation has. I recognize that everyone who's on this call, there's always so much information, whether we're talking about types of interventions, or studies that we're funding, or research that we're funding or investments we're making, or what's happening in the community and events that are taking place, so on and so forth. There's always so much that is going on, and we're here to be a good guide, and a good shepherd, I guess, if you think of it that way, in terms of how to navigate this complex arena.

I also recognize that when you're personally affected, or your family member's affected, it can be quite alarming, and you're not exactly sure where to go. And so, the first thing that I want to make sure everyone on this call recognizes is that we're here to help, not just in terms of driving the research, which we're pretty good at, in terms of moving things forward, but we're also here to help in terms of being able to help navigate this space for you, and with you as a partner.

And so, the first stop is always to check to see what's happening locally within your community. At our website, there's a terrific Find a Chapter locator, and you can punch in your location and identify what resources exist locally. We can help identify, if you need, to Amy's point, if you need to get in touch with a different medical provider. Let's say the ophthalmologist, optometrist that you're working with isn't an expert in inherited retinal disease, and you need to get in touch with someone else, we can help make those introductions, and connect the dots for you.

As well, there are so many clinical trials, 50 clinical trials taking place right now in our space, and we keep our website up to date with a clinical trial pipeline where you have the ability to look and locate every clinical trial that's happening by disease state, and who the sponsor is, where it's taking place, all the information. And even with all these resources, we do recognize that it can be overwhelming

sometimes. And so, at any time, if you want to get in touch with one of us here at the Foundation, you can always contact us directly at info@fightingblindness.org.

So, with that, I know we're at the top of the hour. Oh, one other thing. Sorry, someone just asked a question. "What happens if I have a question outside of the inherited retinal disease space? Let's say I'm having an issue with my cornea, or corneal problems, or glaucoma." I would still encourage you to send us a note at info@fightingblindness.org. We're very close with many other organizations and world experts even outside of inherited retinal disease, and we can always point you in the right direction, and potentially get you on the right path. So, if you have any question related to anything, and even whether it's in our space directly, or maybe right next door to it, don't hesitate to reach out and we'll do everything we can to point you in the right direction.

So with that, as we wrap up today's call, I really do want to just reiterate our appreciation for your participation today, your ongoing support. I urge you to consider the call to action that Jeff highlighted earlier in the call, which is to get involved. By leveraging the combined power of our community, and coming together, we really can achieve great things. That's the only way we've been able to achieve what we have over the last 52 years, which is pretty significant. And so, it's really a matter of everyone that is involved, and touched by this area of the ophthalmology universe to get involved, and help to play a role in advancing our mission.

We definitely welcome feedback and suggestions related to this webcast, or really anything about the Foundation in general, and you can reach us anytime at that email address, again, which is info at fightingblindness.org. And as always, you can learn more at our website at fightingblindness.org. So, I'm going to turn it over now to Chris just to wrap things up for today on the call.

Chris Adams, Vice President, Marketing & Communications:

Thanks, Jason. We'd like to thank everyone again for joining us for today's call. And as a reminder, there will be a transcript, and recording of today's call within the next week available on our website, at fightingblindness.org. And also, be sure to follow us on social via Facebook, Twitter, LinkedIn, Instagram, and TikTok to

stay informed on the latest news, and activities from the Foundation. You can like and share posts to help spread the word. And again, as Jason mentioned, if there's any information, or any assistance you need, please send an email to us at info@fightingblindness.org. Thank you, and have a great day.