Foundation Fighting Blindness
***Insights Forum* Transcript**
August 12, 2022

Ben Shaberman, Senior Director, Scientific Outreach:
Good morning and welcome to the Foundation Fighting Blindness quarterly Insights Forum. I'm Ben Shaberman, senior director of scientific outreach at the Foundation, and we appreciate you joining us for today's call. Before we get started, I would like to briefly review a few details for the call. Currently, all participant lines are in listen only mode with no video. Today's conference is being recorded and is available with closed captioning. To activate the closed captioning, please select the live transcript option located at the bottom of the Zoom interface, then select show subtitles.

Please note that on today's call, our speakers do have their video live. However, all of their comments will be provided verbally and there are no slides. If you're using a screen reader, please be aware that the controls are at the bottom of the Zoom interface. This control bar may collapse when it's not in use. If you prefer to prevent the controls from auto hiding, go to settings within the Zoom platform, select accessibility, and then select always show meeting controls. It might be helpful to maximize your window and navigate by using the tab key. Additional buttons and settings are available by pressing the alt key. During the call, you may ask questions through the Q&A and chat features, or by sending an email to info@fightingblindness.org. We will address questions toward the end of the call during the Q&A session at which time additional instructions for asking questions will be provided. I would now like to turn the call over to Chris Adams, our vice President of Marketing and Communications.

Chris Adams, Vice President, Marketing & Communications:

Thank you, Ben. And good morning, everyone. Thank you for joining us today. I would like to welcome everyone to the quarterly Insights Forum webcast. We are pleased that you joined us today for our updates on a wide range of strategic

initiatives here at the Foundation Fighting Blindness, and to learn more about the research and development progress within our broader community. This is an exciting time here at the Foundation, as we've made several leadership transitions that were announced in June, Jason Menzo was promoted to Chief Executive Officer of the Foundation, having previously served as President and Chief Operating Officer. Dr. Rusty Kelley was promoted to Managing Director of our venture arm, the RD Fund. Dr. Ben Yerxa, who served as CEO of the Foundation since 2017 has transitioned to the permanent CEO role of Opus Genetics, the first spinoff company internally conceived and launched by the RD Fund to further the Foundation's mission.

For today's agenda, I will share an update on marketing initiatives and engagement within the community. Our Executive Vice President of Corporate Development and Chief Business Officer, Peter Ginsberg will provide a summary of noteworthy fundraising and corporate sponsor initiatives along with the highlights of our Fiscal year 2022 financial performance and 2023 budget plans. Dr. Claire Gelfman, our Chief Scientific Officer will provide an update on our 2022 and 2023 science funding, including recently announced new awards. To wrap up our formal comments, Jason will highlight our near-term strategic priorities for the Foundation. After all our formal comments, we will have a question and answer period. And at that time, we will repeat the instructions on how to ask questions. As mentioned, this call is being closed captioned, and a replay and fully accessible transcript will be available on our website in the weeks ahead. We welcome your feedback or suggestions related to this webcast or the Foundation in general. And you can reach us anytime by emailing us at info@fightingblindness.org. And as always, you can learn more at our website fightingblindness.org. I am now pleased to hand the call over to Jason.

Jason Menzo, Chief Executive Officer:

Thank you so much, Chris. And good morning, everyone. It's really wonderful to be with you all today. As you know, we've had some big news just over the last couple of weeks, and I'm excited to share more details about where we're heading as an organization a little bit later in the call. With that said, I do want to

start today's call by recognizing the tremendous progress we've made towards our mission over the past five years under Dr. Ben Yerxa's leadership. Everyone at the Foundation, of course, wishes him and the team at Opus Genetics a ton of success as they continue to further our mission collectively. I'm really honored to carry the momentum forward as we continue our pursuit of our mission, and I'm excited to work closely with Dr. Rusty Kelley who will be leading the RD Fund and our entire executive and leadership team.

We really are all so passionate about this mission and committed to making a meaningful difference in the lives of those who are in our community. We're working to do this with a continually improving operational structure powered by our dedicated staff and volunteers all across the country. And in addition, the Foundation has the invaluable insight and oversight and input from our Board of Directors, our Scientific Advisory Board, the RD Fund Board, of course, all in the scientific community. Our team has identified four strategic priorities that I'm going to summarize now, and then elaborate in more detail at the end of the call. But I wanted to start the call off by listing these four strategic priorities as we go forward.

Number one, accelerate investment in the best research across all stages of development. Number two, increase awareness amongst a broader audience to drive additional fundraising. Number three, ignite our community for deeper engagement and participation. And then number four, to be a model non-for-profit citizen. We want to be a leader in not just our field, but really across the spectrum of non-for-profits in terms of how nonprofits can operate and impact their respective missions. This really is a significant advancement and a significant time for our field, and I'm inspired to serve all of you in this new role. Again, I'm going to go deeper on those four strategic priorities a little bit later in the call, but for now I'm pleased to hand the call back over to the team to provide updates on the marketing, financial and scientific funding initiatives that support our strategic priorities. And we'll be back in a few minutes. So with that, I'm going to turn it back over to you, Chris.

Chris Adams, Vice President, Marketing & Communications:

Thanks, Jason. I'm pleased to have the opportunity to share with everyone on the call today the many marketing and communication initiatives we have underway.

We are focused on increasing the outreach, support and engagement within our community and beyond. These efforts will ramp up in a big way this fall, as we honor Blindness Awareness Month in October. We are launching a marketing campaign called Share Your Vision, which will empower individuals and their loved ones who are impacted by blinding retinal diseases to share their vision loss journey through written word, audio, or video. Thank you to our campaign co-chairs, Christine Exley and Drew O'Brien.

With this campaign, we are providing unique tools and templates on our website to make sharing stories simple and easy. This includes a unique vision viewer, which will provide a visual representation of how your site is impacted depending on the type of retinal disease you have and the degree of vision loss. The campaign promotion will begin in September. Everyone will be able to start sharing their stories on social media starting in September, and throughout the month of October using the hashtag, share your vision.

In addition, we will select a variety of these stories to be featured on the Share Your Vision website at fightingblindness.org/shareyourvision. An email will be distributed next week to provide more information about this exciting campaign. There will also be special days highlighted during the month of October, including World Sight Day on October 13th, and White Cane Day on October 15th. The culmination of all the October activities will be the next installment of our popular livestream music series, Music To Our Eyes, which will be on October 27th, featuring singer and songwriter, Mark Erelli.

Another feature on our website is our do-it-yourself fundraising program called Raising Our Sights, which enables anyone to fundraise for the Foundation anywhere at any time throughout the year. Our community members can leverage creativity, passion and connections by designing an online campaign, hosting an athletic event, fundraiser, creating a tribute or using a special occasion, such as a wedding or anniversary as a fundraising opportunity, whether it's a small personal fundraiser or a large community event, this support is vital to the Foundation. Every dollar matters in funding research to finding meaningful treatments and cures. You can find out more information on our website by visiting fightingblindness.org/raisingoursights, or by finding the link on the ways to give section under DIY fundraising.

We also hosted several special events over the past year that raised more than two million dollars. Most recently, we hosted our 22nd annual Microsoft Scramble for Sight Golf Tournament represented by RE/MAX in July at the beautiful Sanctuary Golf Course in Colorado. The event generated more than $300,000 and included an interactive generational panel discussion that raised awareness of the meaningful and important work that the Foundation Fighting Blindness has done. Thank you to the event co-chairs, Scott Burt and Sherry Gruenberg.

For more information on the many social events that we have planned this Fall and into next year, please select the Foundations and events tab on the website at fightingblindness.org. Our website has numerous other resources of helpful information, including highlights and recordings from our recent Visions conference and the Eye on the Cure Podcast series hosted by Ben Shaberman, which provides science information, news, insights from the world of vision and retinal diseases. We are always adding new content to our website, so make sure to visit our site often. I would now like to turn the program over to Peter Ginsberg, our Executive Vice President of Corporate Development and Chief Business officer. Peter.

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

Thanks, Chris. So I'd like to begin the discussion of our funding sources and financial snapshot by highlighting the progress we have made in our multi-year Victory For Vision campaign. Last year, as you know, we set a bold fundraising goal for this campaign and that objective was to raise $50 million over the next five years, which is in addition to our regular annual fundraising. I am quite delighted to report that through the tremendous efforts of our campaign volunteer leadership and staff, we've already surpassed that original $50 million goal. We now have more than $53 million dollars in commitments for the Victory For Vision campaign and have expanded the campaign target from $50 million all the way up to $75 million dollars. We all want to express our tremendous gratitude to our campaign co-chairs David Brint, Robert Heidenberg and Marsha Link, and also our volunteer leaders across the country and the globe. This level of funding is critical in continuing the rapid pace of developments across early academic research, all the way to clinical testing and regulatory approval.

Now, in addition to our individual donors, we continue to collaborate with corporate sponsors to gain funding for important initiatives. We proactively connect with leading and emerging companies in our field and beyond that help fund various Foundation initiatives. Along these lines, we're pleased to announce new outreach partners, EyePoint Pharmaceuticals and Lexitas Pharma Services. EyePoint is developing therapeutics for patients with serious eye disorders leveraging its proprietary Durasert technology. EyePoint is currently in Phase 2 testing for sustained delivery of anti-VEGF treatment, initially targeting wet AMD. Lexitas is a full service ophthalmic focused solutions company providing clinical trial and medical strategy services to pharma and biotech companies that are developing ophthalmology products. We greatly appreciate the support of all of our corporate sponsors, including our newest outreach partners, EyePoint and Lexitas.

Changing gears, our signature VisionWalks are another very important part of the Foundation's fundraising and awareness building. In the fall there will be 13 walks in communities across the country with 20 more planned for next spring. Over the past year, we hosted 37 in person walks across the country, raising over $3.4 million dollars and involving more than 8,000 attendees and 720 teams. We can't wait to see you at one of our VisionWalks this fall.

Now I'd like to provide a brief summary of our financial position and our budget outlook for the coming year. Recall that the Foundation operates on a Fiscal year that runs from July to June, so we just completed our Fiscal 2022 on June 30th. Our audited financial statements will be available this fall on our website in the About Us section under Financial Reporting. But in the meantime, I'm pleased to provide the following summary based on our preliminary unaudited financial results for Fiscal 2022.

I'm excited to say, we expect to exceed both our $27.7 million budget on unrestricted revenue and our $9.6 million budgeted net fundraising surplus for the year. So well ahead of our expectations for both revenue and surplus. And that most importantly enables us to fund all of our prior research commitments and also fund the new research commitments that Dr. Claire Gelfman will highlight later in the call.

For Fiscal 2023, so looking ahead to the current Fiscal year that we just started, we're targeting $31 million in unrestricted revenue against $18 million in operating expenses for a $13 million net fundraising surplus, and you'll note that's significantly above the Fiscal 2022 budgeted numbers. And importantly, that net fundraising surplus of $13 million that we're budgeting for Fiscal 2023 will go to support new research funding.

And I also will note that the unrestricted revenue that we're budgeting for Fiscal 2023 is above the revenue that we had exceeding the pre-pandemic levels. At the same time as we're generating strong revenue growth that we can put toward important new research, we're continuing to focus on streamlining operating expenses and becoming even more efficient. For example, we've been able to reduce our office rent and related expenses, as we have many team members continuing to work from home offices. I will conclude by reiterating that it's only through the crucial support of donors, sponsors and funding partners that we're able to fund this cutting-edge research. I'll now turn the call over to Claire Gelfman, our chief scientific officer, who will provide more detail about the research that we're funding. Claire.

Dr. Claire Gelfman, Chief Scientific Officer:

Thank you very much. It's really great to be here to have everyone on the call today. Based on the very strong support that Peter just mentioned, the Foundation is able to fund a very diverse portfolio of emerging therapies to address the entire spectrum of inherited retinal diseases and dry AMD for all patients affected, regardless of the mutated gene causing their disease or the severity of their vision loss. The level of interest and need for funding remains very high though.

We are currently funding 84 active grants being conducted by more than 100 research investigators at 65 institutions, eye hospitals and universities. During Fiscal year 2022, the Foundation approved 23 new research programs, totaling 15 million dollars of funding.

As part of our 2022 funding, we recently announced the recipients of our Individual Investigator Research awards based on a rigorous review by our scientific advisory board, we are funding eight new awards for Fiscal Year 2022. These awards are going to researchers at Baylor College of Medicine, Duke

University, Johannes Gutenberg University of Mainz, Harvard Medical School, Oregon Health and Science University, the University of Massachusetts Amherst, the University of California San Diego and University of California San Francisco.

The researchers at these institutions are working on a range of cutting edge approaches to understanding and treating inherited retinal disease. This includes research on Usher syndrome, Stargardt, EYS-associated retinal degeneration, lebers congenital amaurosis, human cone dystrophies, as well as genomic data to identify novel IRD genes and mutations, as well as cellular proteostasis to treat IRDs.

In addition, we are funding two program project awards. One award was granted to UCL Institute of Ophthalmology, which is investigating the novel disease mechanism for autosomal dominant retinitis pigmentosa type 17 and exploring therapeutic approaches. The second award went to the Foundation Voir et Entendre, which is working on the disease pathogenesis and treatment solutions for vision loss due to retinitis pigmentosa associated with Usher syndrome type 1B.

Earlier this year, we announced the 2022 award recipients of our Translational Research Acceleration Program, also known as TRAP. These awards are targeted to accelerate the movement of particularly promising preclinical research towards the clinic. One of the highlights of the year is when TRAP award recipients present their research to the IRD community. This year, those updates will be presented at the TRAP kickoff webinar on August 29, 2022 at 10:30 AM Eastern time. That is open for anyone to attend.

As we look forward, our Board of Directors approved our Fiscal Year 2023 science budget for new research funding of $23.5 million as part of our five year science strategic plan. And our 2023 TRAP application cycle is already underway. The request for applications was released on April 25th of this year. There were 53 letters of intent submitted in June of 2022 that are currently being reviewed by our Scientific Advisory Board. The applications with projects with the highest potential to advance in the preclinical research and development process will be invited to submit full applications, which will be due in October of 2022. After review, the Advisory Board will be making funding recommendations to the Foundation's Board of Directors in the spring of 2023.

As we recognize the research awards made over the past year and look forward to new ones in the coming year, we especially want to recognize the very generous support of our funding partners, including the Diana Davis Spencer Foundation, the Free Family Foundation, The George Gund Foundation, the Nixon Visions Foundation, and Save Sight Now.

As I wrap up, I'd like to highlight one recent development in the clinical research landscape. Apellis Pharmaceuticals announced that the US FDA has accepted and is now reviewing its new drug application for the potential approval of intravitreal pegcetacoplan, the company's therapy to treat geographic atrophy. There are currently no approved treatments for geographic atrophy, which is the advanced form of dry age-related macular degeneration. Pegcetacoplan is an investigational targeted C3 therapy designed to regulate excessive activation of the complement cascade, which is part of the body's immune system, which can lead to the onset and progression of many serious diseases. The FDA's review of pegcetacoplan is currently ongoing with a response of approvability due in late November this year. And we look very forward to tracking the outcome of this potential new therapy.

As you can tell, there is an exponential amount of innovation, new developments, and progress across our entire range of research funding. The awards that we made this year will fuel our future achievements and ability to ultimately help everyone diagnosed with both inherited retinal diseases and dry AMD, regardless of their gene mutation and degree of vision loss. I'm now pleased to hand the call over to our CEO, Jason Menzo.

Jason Menzo, Chief Executive Officer:

Thank you so much, Claire. And thank you to everyone on the team for the comments and the overviews so far in this call. And again, thank you to everyone for joining us today. I want to make a few comments really describing the state of where we are as an organization right now, and more importantly, where we're going. In the 51 years since the Foundation was formed back in 1971, this organization has actually been through many iterations and really some really important key milestones. As you may know, we began as the RP Foundation and we operated primarily as a volunteer led organization funding a few select labs,

really focused on the discovery of the first several genes known to be related to what we now know as inherited retinal diseases.

Over the years that followed, we evolved and we grew into our national footprint with staffing coast to coast. We launched our VisionWalk program, which Peter summarized earlier in this call, we formalized our national grassroots network of over 40 chapters. And then in recent years, we've seen some of the early fruits of our labor with the approval of the first gene therapy for an inherited retinal disease in LUXTURNA. This track record of success, beginning with a blank slate and then leading to a treatment for a target condition is really, truly remarkable, but we all recognize that there is still so much work to do, we're just scratching the surface. The Foundation has played a role in identifying the more than 300 known gene mutations that contribute to IRDs. And based on our global network of scientists, physicians, and patients, we're poised to make even greater contributions in the years ahead.

Today, we fund the best labs all over the world with hundreds of millions of dollars invested in this mission to date, and we are driving the mission forward faster than ever. We host one of the largest patient databases in the space called My Retina Tracker. We have over 21,000 members in My Retina Tracker today. And through this registry and the accompanying genetic testing program, we're helping to identify the gene causing variants for thousands of affected individuals every single year. And as you know, we also recently launched our venture arm called the RD Fund, or Retinal Degeneration Fund to spell it out. And through the RD Fund, we invest in early-stage clinical programs to accelerate them towards regulatory approval and eventually commercialization. Really a lot has been accomplished in the first 50 years of our life. But again, there is still so much work to do, and we recognize that.

As I start my tenure in this new role, I'm super excited to think about what we can accomplish next. What can we do in the next five years? What can we accomplish in the next 10 years? And as I've said before, the rate of discovery and development has become exponential, so we should all raise the bar in terms of what we can expect out of this field and what we can contribute as an organization in the years to come. On today's call, I'd like to use this as an

opportunity to share four strategic priorities that represent our near-term opportunities here at the Foundation.

Number one, our first priority is to accelerate the investment in the best research across all stages of development. We recognize that in order to drive our mission, the first and most important thing that we can do is accelerate our investments in the range of research programs that are driving it. In the year ahead, we're going to be evaluating all of our programs and measuring them against the gaps in the field. We're going to begin development of a new long term scientific strategic plan and aim to put the maximum amount of resources available towards the areas of greatest impact.

Number two, we're going to increase awareness amongst a broader audience to drive additional fundraising. To fund these initiatives that we just outlined in our first priority, we of course need to engage new audiences and increase the funds available for investment. While the Foundation is benefited from the generosity of many of you on the going to call today and all of the tens of thousands, hundreds of thousands of constituents who have funded us to date, to catapult our fundraising to new levels, we must engage new audiences, and we recognize that.

As we've reported repeatedly on these calls over the past few years, we have increased our exposure in the marketplace through our public service awareness campaigns, our targeted advertising on television and social media and many other strategic initiatives. And we plan on driving these efforts even harder with the aim of welcoming more people under the tent to help us win. We plan to make a major announcement in the weeks ahead related to our Share Your Vision campaign that Chris described a little bit earlier today. And we plan for this to be a really successful initiative come this October.

Our third priority is to ignite the community engagement and participation of the blind and low vision community. Active members of our community are the lifeblood to our organization and help feed our accomplishment of our mission. In order to sustain and magnify this energy, we need to actively engage everyone in our community. We're committed to developing our chapter network, which currently exists of 40 volunteer led chapters, we call it Lulie's Next Chapter for Light & Vision. And this important initiative is expanding. We're adding the

number of people that are engaged and involved in the current chapters, and we're looking to expand the number of chapters with a goal to grow our chapter network to greater than 60 chapters in the next couple of years. It's going to be really important for us to engage our blind and low vision community across the country coast to coast.

And the fourth and final priority that I want to highlight today relates to our commitment to being a model non-profit citizen. As a mission driven organization, we strive to direct the vast majority of our funds towards mission related initiatives, we call them programmatic initiatives. That said, we aspire to also be a model in our field, and not just meet that bar, but to be a shiny example of what great looks like as a non-for-profit. We pride ourselves in transparency and accessibility and diversity and equity and our strong collaborators with others in the space.

Actually, one of the ways that we strive to ensure good communications is by hosting these quarterly Insights Forum calls. Believe it or not, today is actually our 15th Insights Forum. And since we started these calls, which seemed like a second ago, this just seems like such a new thing, but we've already been a couple of years into it. We started these calls in November of 2018, we've had over 6,000 participants engage with the Foundation through these Insights Forum calls. We've had guest speakers ranging from top researchers in the field, to corporate executives, to government and community representatives. We even had a sitting member of the House of Representatives join us as a guest at one point.

Underlying all of these strategic priorities is our mission, which has remained the same, and it is really our north star, it's to drive the research that provide preventions, treatments and cures for people affected by retinitis pigmentosa, age-related macular degeneration, Usher syndrome, and the entire spectrum of inherited retinal diseases.

I really do look forward to working with all of you in collaborating globally with our researchers, medical professionals, corporate sponsors, investors, legislators, regulators, the list goes on and on, and importantly, our other non-for-profit and industry organizations.

In closing, for today's call, I want to ask everyone on the call to do me a favor. If what we do matters to you, take a moment in the next day and introduce

someone new to the Foundation. Share with them, our website. Make a post on social media. Go to our Facebook page. Share the link to this call today. If what we do matters to you, take the opportunity to help us get the word out.

With that, it's 11:30 here on the East Coast, and we're going to open things up for questions. We're going to have a good half hour for questions today. I'm going to pass the baton back over to my friend, Ben Shaberman, to give the instructions for asking questions.

Ben Shaberman, Senior Director, Scientific Outreach:

Thank you, Jason. There are several methods for asking questions. First, you can access the Q&A and chat features located at the bottom of the Zoom control bar and type in your questions. Second, you can ask questions verbally. And to do so, please select the hand raising function on the menu bar at the bottom of the Zoom interface, and we'll provide you with instructions to unmute yourself. And third, you can submit your questions via email to info@fightingblindness.org. And please note that if there are questions that we aren't able to answer on today's call due to time constraints, we'll follow up with you directly via email over the next week or two.

Jason Menzo, Chief Executive Officer:

Thank you so much, Ben. And while we're compiling questions, I would like to let you know that in addition to our speakers from today's call, we're pleased to have Dr. Todd Durham join the Q&A session. Todd, I welcome you to come off mute and turn your camera on. Todd is our Senior Vice President of Clinical and Outcomes Research. I'm also excited to have Dr. Amy Laster who's our Vice President of our Science and Awards programs, and really drives the strategy for a lot of the programs that allow us to meet our mission. And with that, let me actually pose the first question to you, Dr. Laster. Amy, we talk a lot on these calls about our programs and our awards and how we feed the field through funding the best science, maybe you could take a minute and share a little bit about our grants and awards program and how we go about that process.

Dr. Amy Laster, VP, Science & Awards Programs:

Thank you, Jason. Hello to everyone, glad to be here. I'll just start by saying this year we are employing eight funding mechanisms that will promote research and

clinical studies global in our key areas for this field. And these mechanisms include developing clinician scientists, creating animal models and building team science. We are very proud that, when we say we select research that is after a rigorous review process, well, what we really mean by this is that we have a global expert Scientific Advisory Board of more than 60 members. These individuals are very key in helping us to determine what are the key gaps in the field and where should we be prioritizing our area, our funding, and the funding that those that are on this call help to provide.

And so with their input, they're reviewing over 100 research proposals, I know over the last few years, and helping to make these selections of what fits into our priority. They're guiding the funding mechanisms that we have. So things from therapeutic approaches, like how do you address disease by treatments? As well as, what are the basic information that we need to know? As Jason mentioned, gene discovery. We need to understand what things look like in the clinic. And so these are some of the guidelines that they use in our process. We have two stages of the process. There are pre-proposals that they review and then there are full applications that they review, and that takes about anywhere from six to nine months for that process to happen before those funding recommendations are made. And I'll stop there, Jason.

Jason Menzo, Chief Executive Officer:

Thank you so much, Amy. I do want to remind everyone you can either chat questions in or you can ask actually use the Q&A section in the Zoom, and we'll come back to you actually in one more minute, Ben Shaberman, maybe you can reiterate the detailed questions. But before we do that, Todd, I want to actually ask you, one of the most important initiatives that we have at the Foundation, we talk a lot about it, is the My Retina Tracker Registry and the genetic testing program. We often get a lot of questions about, why does it matter? Why does it matter to know what my gene is? How do I go about getting tested? If I was tested 10 years ago and they didn't find a gene, when should I get tested again? Maybe you could speak just at a high level about some of those topics.

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

I'll remind everyone attending today about what My Retina Tracker Registry is. This is a study, it's an observational study to help us understand what are genetic causes of disease and what are their manifestations, particularly for people with inherited retinal diseases. And it also gives parents of affected children the opportunity to register and tell us about their child as well. My Retina Tracker Registry is used many times to link individuals with the IRDs to research studies. This is one of the great advantages of joining the study, as you can participate in that program.

And as Jason alluded to, one of the features of this program is we have a program that enables people to access a genetic test and counseling session at no cost to them. This is called the My Retina Tracker Genetic Testing program. It is in sponsorship and collaboration with Blueprint Genetics and InformedDNA. If you haven't been tested in a while and you had inconclusive results, or you've never been tested for your IRD to understand what your genetic cause of disease is, this program is open to your ophthalmologist, your healthcare provider. If in their state or their location, they're allowed to order a diagnostic test, they're able to access this program. So just ask for My Retina Tracker Testing Panel. And the technology and the ability to detect disease causing genes does change over time. It doesn't change every few months, but it does change over time. And the criteria that we use with our partner depends on what those changes have been. So those requirements change over time, but that is part of the program information that your provider will find on the website with Blueprint.

Jason Menzo, Chief Executive Officer:

Thank you so much, Todd. We have a bunch of questions that are being chatted in now into the Q&A session. But, next I actually wanted to address one of the questions that came in through the chat to you, Dr. Gelfman. Claire, the question was, when could we expect a treatment for retinitis pigmentosa? And maybe you can use this as an opportunity to talk about not just the development pipeline for RP, but really the difference between approaches that are gene specific and gene agnostic. Todd was just talking about the importance of understanding your gene,

but there's obviously other approaches that are gene agnostic and it would be great to hear from you on that.

Dr. Claire Gelfman, Chief Scientific Officer:

Thank you, Jason. This is Claire Gelfman. You're right, it's important, if possible, to get genetic testing to identify the cause of mutation that accompanies your clinical diagnosis from your physician. That's not always possible, so in addition to the more gene specific approaches where companies and researchers are giving back an unaltered copy of the gene in order to restore protein function, another option is going to be amenable for those who do not necessarily know their causative gene. And that's really all about learning what's happening at the level of the cell with respect to a degenerating photo receptor that causes vision loss. And so the example that I like to give is that you may have 10 people in a room with a different mutation causing the retinitis pigmentosa, but what's similar is the vision loss due to our rods and our cones that are degenerating.

I'm happy to report that these gene agnostic approaches are potential therapies that are being developed that are not dependent upon knowing your cognitive gene, and instead, are all about fixing, if you will, that cellular process that's common regardless of whether or not you know your specific mutation. And there are a lot of different approaches that are being evaluated in that realm. One that we hear a lot about is optogenetics. It's a scary word, but essentially it is giving the ability to respond to light, that normally is something that happens at the level of our photoreceptors. If our photoreceptors are degenerating, what this process is doing is giving that ability to respond to a part of our retina that's not degenerating. And that's important because, and this answers another question from the chat, that if you have late-stage photoreceptor degeneration and your photoreceptors cannot be amenable to any gene-specific therapy that relies on a viable cell to receive the therapy, these agnostic approaches like optogenetics are not necessarily dependent upon that, and instead can be a viable treatment for late-stage photoreceptor degeneration.

Jason Menzo, Chief Executive Officer:

Excellent. Thank you so much, Claire. I'm going pass the next question in a minute to Ben Shaberman to discuss what's happening in the field as it relates to

achromatopsia . But before I do, I'm going to actually answer two questions related to various topics that I think are really important. Number one, there was a question that was chatted in, and this is Jason Menzo by the way, there's a question that was chatted in about progress with LCA5. And we've talked a lot over the last couple of Insights Forums about LCA5 in particular, which is a specific gene variant that causes issues within LCA, obviously.

And we launched, out of the RD Fund, the first spin out company, actually just over a year ago called Opus Genetics. The lead program that is closest to clinic within Opus Genetics' portfolio is a treatment for LCA5. The company is moving rapidly towards a clinical trial, and we're optimistic that they'll actually enter the clinic either at the very end of this calendar year or the very early part of next year. And so Opus Genetics is a terrific example of how as a Foundation we can influence not just our funding in preclinical or translational, but also through the RD Fund, really put resources behind companies that are pursuing our mission. And so that's an important highlight. And of course, you can go to their website at opusgtx.com to learn more about their programs.

The second question that I'm going to address was related to a news announcement that came out yesterday from a company called ProQR, Pro-Q-R, it's pronounced procure. And announced yesterday that ProQR was going to be partnering out their ophthalmic programs, including a program for CEP290, sepofarsen. And the second program that we actually were collaborators with them on related to a particular form of Usher syndrome type 2A. And so as a publicly traded company, ProQR made this announcement yesterday that they're going to be partnering these two programs out. We're really close with the team at ProQR. We're excited to help them find a new collaborator that will hopefully enter the field soon to take these two programs forward. And it's important for the field, that programs that are in the clinic continue to get the funding and continue to move forward. And we're really, as I mentioned, close with the company and anticipate, hopefully in the near term, a new collaborator to enter the field and to continue moving these assets forward.

And then finally, the same individual who asked that question of ProQR asked, what percentage of our funds go towards operational costs versus research funding? I'll tackle that one as well, it's really simple. Our aim is to move closer

and closer to the 80% mark of programmatic spend, most recently we've been in the 75% range, where 75% of our funds spent go directly to programs, meaning the research or public health and education. But we have a goal to continue to move that bar even higher in the years ahead, so hopefully that answers that question. With that said, we've got about 15 minutes left. I'm going to pass the mic over to Ben Shaberman to talk a little bit about what's happening in the field with regards to achromatopsia.

Ben Shaberman, Senior Director, Scientific Outreach:

Thank you, Jason. And just for our participants to understand, achromatopsia is a condition that causes what we call day blindness. It can be caused by anywhere from, well, in a general range, about one of six different mutated genes. And what happens in people with achromatopsia is they have extreme sensitivity to light. So being in lighted conditions, being out in sunshine, even being in a bright room indoors can be very uncomfortable. Achromatopsia also causes significant issues with visual acuity and color perception.

Now, there are two genes, I mentioned there are about five or six genes, each of which when mutated can cause achromatopsia, there's been gene therapy development for two specific achromatopsia genes, CNGA3 and CNGB3. Mutations in these genes cause a vast majority of the cases. Now the company, AGTC, has done gene therapy development for both CNGA3 and CNGB3. And at this point, AGTC has decided to continue on after some encouraging results in Phase 1/2 for CNGB3 to move forward in developing a CNGB3 gene therapy. So stay tuned for further information on that. There are a couple of other organizations that have been working on gene therapies for CNGA3 and CNGB3, but they have not reported out results, it's really been AGTC that's been public with information on their gene therapy development for achromatopsia.

Jason Menzo, Chief Executive Officer:

Thank you, sir. Next Amy, I'm going to come back to you, Dr. Laster. There was a question, we talk a lot about, even on this call, we've talked a lot about gene therapy and gene agnostic therapy, but there are obviously other strategies as well. And someone had asked specifically around what we're funding in the field of cell therapy. And I'm wondering if you could speak to that?

Dr. Amy Laster, VP, Science & Awards Programs:

When we talk about cell therapy, cell-based therapy, you may hear us talking about regenerative medicine. We're talking about these strategies that's going to provide some functional rescue or replacement of our degenerating or dead retinal cells and disease. These can lead to either slowing or maybe prevention of vision loss. We're also optimistic that these strategies may also lead to restoration of some loss vision.

What we have been funding and currently fund is research around a few strategies. At the University of Wisconsin, there is research to develop these very young type of photoreceptor cells. They're created from patients, and the intent is to replace the damage at areas of the retina and using methods of cell transplantation. And so by using cells that are coming from the patients, then there is less tissue rejection than if you were using cells from a different source. And other institutions that we work with such as the University of Wisconsin, and some of the campuses at the University of California system, researchers are working to define methods that will coerce one type of a retinal cell to develop into a photo receptor, and so this is called reprogramming. This is just another strategy to replace damaged cells. In general, we also look at investigators whose labs are working on methods that will improve cell rejection and inflammation. These are two common things that can often impede the successful kind of cell-based therapies that we would like to see.

Jason Menzo, Chief Executive Officer:

Excellent. Thank you much, Amy. And next I'm going to come back to you, Todd. So oftentimes, this is actually a really good education, I think for all of us, we hear of these things like Phase 1, Phase 2, Phase 3, IND, NDA, a lot of the jargon that goes along with clinical development of a program. Can you just give a brief overview, there's a question really specifically about how much time is a program in phase one or how much time does the program typically take to move from phase one to phase three, a pivotal, for example, maybe you could just give a brief overview of that process in the timeline, and then we'll go on to the next question.

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

As an example of how long this process can take, despite all of our shared urgency over the final outcome of a useful product being approved, it can take many years. In the case of LUXTURNA, from the point they filed an investigational new drug application, that is essentially the request to proceed information to the Food and Drug Administration, it took Spark Therapeutics 12 years in the development of that successful product. So the entire span can take a long time.

And there are many variables here, as the question indicates or suggests. In the case of a gene therapy, oftentimes, like LUXTURNA, what we're seeing are one year Phase 2 or Phase 3 studies, following individuals for one year. And in the case of LUXTURNA and other gene therapies, we're seeing after that one year crossover to treat the second eye and see the effects of that. So oftentimes those studies are about two years in duration of follow up at a minimum. Then you have to think about how long does it take to recruit the individual to participate in that study? I would say for a relatively small program, it could be something like two and a half years to three years at that stage of development. This is a slow and deliberate process because we're dealing with human health, but it also needs to be rigorous and well done.

Jason Menzo, Chief Executive Officer:

Excellent. Thank you, Todd. Peter, I'm going to actually address this next question to you, and I'll set it up. I spoke a few minutes ago about the program ratios. There's a question, we have this aspiration to be a model non-for-profit citizen, and maybe you could speak briefly about what goes into our program ratio. So when we say that we're in the neighborhood of 72 -75% currently of funding going towards programs with the aspiration of continuing to even grow that, what do we mean when we say we're funding our programs as our mission related expenses, as compared to other things? Maybe you could speak to that?

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

Happy to do so, Jason. So program and spending ratio, the formula is research and public health education related expenses divided by total expenses. So when Jason talks about a program spending ratio in the 75% range, that means that our

science research and public health education related expenses are about three quarters of our overall expenses. And I should point out that the charity monitoring groups like Better Business Bureau, GuideStar, they look to see those program spending ratios be above 65%. We tend to look to be well above 70% with an ultimate goal of moving north toward 80%, so well above the charity monitoring groups guidelines, but we still aim higher.

Jason Menzo, Chief Executive Officer:

Excellent. Thank you, Peter. And again, everyone on this call, we're making this commitment to you today that our objective is to be a non-for-profit model. And that just that bar, the minimum bar of 65% is not where we want to be, we want to continue to raise the bar and be a great model, an example of what good looks like with regards to efficiency and in focusing our resources towards why we're here, which is to move our mission forward. It's really important.

Next I'm going to come back to you, Claire, Dr. Gelfman. We had a question, and there's a couple different questions that are in the same neighborhood, which is related to, we talk about health of the photoreceptors and what happens when those photoreceptors that have gotten to the point of complete degeneration, and then treatment modalities like optogenetics as a potential treatment for individuals when they get to that state, and I'm wondering if you could go a little bit deeper on both? How do we know when the photoreceptors have degenerated to that point? And then maybe even deeper a little bit about what optogenetics is and why that's an important tool that we're anticipating in the field in the coming years?

Dr. Claire Gelfman, Chief Scientific Officer:

Thanks, Jason. Again, this is Claire Gelfman. So just to elaborate, I think it's important to really appreciate the role of the photoreceptor. So we're talking about rods and cones that are responsible for either our peripheral or our central vision respectively. And when those begin to degenerate, you'll notice, you'll have conversations with your physician that different parts of your visual field will begin to go dark and your physician can really help track if that's coming from your rods or your cones, whose job is to respond to the light coming in and then send a message to your brain so you can interpret the image in front of you.

Whether your vision lost is central or peripheral can really be a sign that the rods and the cones whose job is to again, trap that light and send a message to our brain, if we can't do that due to some genetic mutation, then it's very important that we talk about that with our physician to evaluate potential therapies.

And as I said before, one option is a gene specific therapy. However, if you have a degenerating photoreceptor translation, you either have lost most of your vision, so clearly the function that I was just describing is really severely compromised. The question is, how can we still enable a therapy to restore vision if we don't have those photoreceptors? How can we bypass the photoreceptor function to still get that message to our brain? And that's really what optogenetics is all about.

And to dig a little deeper, Jason, to your question, there are proteins in the photoreceptors called opsins. And the job of the opsins is to trap that light and send that message to the brain. So we literally use gene therapy to deliver an opsin to our ganglion cells, parts of the retina that are not degenerating in order to bypass the photoreceptor to still get the message to our brain to restore visual function, and that's really what optogenetics is all about. There are individuals that have participated in a clinical trial that really, that one question is being answered. And what has been shown is that even individuals with almost no light perception are gaining some visual acuity through bypassing the photoreceptors in that way and to the point where they can actually make out not only objects on a desk, but a crosswalk on a street. So we're really encouraged by this and other ways to bypass a malfunctioning photo receptor.

Jason Menzo, Chief Executive Officer:

Excellent. Thank you, Claire. We've got about three minutes left, so I'm going to have just one final question and then we'll wrap up today's call. But I do want to reiterate that there are many questions that we didn't get to today, but every single question that is chatted in or posted on Facebook or sent to our info@fightingblindness.org or any other way that you've tried to ask a question today, if we didn't get to it on the call, we always follow up with every single individual on every single question in the weeks ahead. So if we didn't get to your question, don't be alarmed, we will follow up with you individually.

The last question, we've talked about gene therapy. We've talked about gene agnostic therapy. We've talked about cell therapy. We talked about optogenetics just now. What we haven't talked about yet, which is another hot topic in the field, and it's something that a lot of folks ask, and there's a couple of questions chatted in today about it, is gene editing and CRISPR. And so Ben Shaberman, I'm going to ask you to just give a quick update on what's happening in the field as it relates to CRISPR.

Ben Shaberman, Senior Director, Scientific Outreach:

Thanks, Jason. So CRISPR, for those of you that don't know, is gene editing. It's a treatment where you're coming in with, what you can think of as a pair of molecular scissors, to just cut out the mutated part of the gene, and in some cases, paste something new in. In some cases we don't. So gene editing, molecular scissors. Excitingly, the company, Editas, several months ago reported some encouraging vision improvements in its clinical trial for people with a common mutation in CEP290, and that mutation causes LCA 10, Lebers Congenital Amaurosis type 10.

So that trial continues to move forward, but there's a lot of great additional CRISPR research going on that's moving toward the clinic. And when I was at a major research conference called ARVO earlier this year, in early May, a research group that's working with Editas reported some exciting results in a lab study for the gene, rhodopsin. Rhodopsin when it's mutated is a common cause of RP and they showed in a large animal model that CRISPR in conjunction with gene therapy was able to restore an impressive amount of rhodopsin function in this large animal. And I have not heard specific plans for Editas to move that treatment into a clinical trial, I want to make that clear, but that large animal study does position them well to do that if they decide that's a prudent move on their part.

Jason Menzo, Chief Executive Officer:

Very good. Thank you, Ben. And let me turn it over to Chris, just to wrap up this call today.

Chris Adams, Vice President, Marketing & Communications:

Thank you, Jason. It's Chris Adams. We'd like to thank you all for participating in today's call. As Jason mentioned, if we didn't get to any your questions today, we'll be following up with you directly via email. As a reminder, there will be a transcript, an audio recording of today's call on our website within the next week. Our website address again is fightingblindness.org. You could also get information from our Facebook, Twitter, LinkedIn, Instagram, and our newly launched TikTok accounts. They are all great resources for learning about the latest developments in the retinal disease space. If there is any other information you need, please reach out to us by sending an email to info@fightingblindness.org. Again, that email address is info@fightingblindness.org. Thank you, and have a great day.