Foundation Fighting Blindness
***Insights Forum* Transcript**
August 24, 2023

Amanda Bement, Chapter Engagement Assistant:

Hello and thank you for joining today's Insights Forum call. Before we get started, I would like to briefly review a few details for the call. Currently, all participant lines are muted and without video. Please be aware that the controls are at the bottom of the Zoom interface. This control bar may collapse when it is not use. If you prefer to prevent the controls from auto hiding, you can use the following keyboard shortcuts to toggle the always show meeting controls options. If you are using a window command, press Alt P, 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, please select the ellipsis or three dots located at the bottom right of the Zoom window, select captions, and then show captions. Please note that on today's call, our speakers do have their videos live. However, all of their comments will be provided verbally and there are no slides.

Throughout the call you will be able to ask questions via the Q&A feature and the chat feature, both are at the bottom of the Zoom window. We will 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 week, so please be 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 Menzo, Chief Executive Officer:

Thank you so much, Amanda. Thank you everyone for joining us on today's call. I'm thrilled to see all the chats from people all over the world and folks joining us live on Facebook as well. We have a terrific call planned today, and I'm really pleased to welcome you all to our quarterly Insights Forum webcast. Our goal for these calls is really to provide updates on strategic initiatives that are happening

here at the Foundation Fighting Blindness, as well as what's happening in the broader inherited retinal disease community, really all over the world.

We have a great lineup for our agenda today. First, Chris Adams, who's our Vice President of Marketing Communications, is going to provide an update on our marketing initiatives and the many ways that we're reaching out and engaging with constituents across the community throughout the U.S. and really throughout the world. Then Peter Ginsberg, who's our Chief Operating Officer, is going to highlight some of the recent notable corporate announcements and sponsorships happening in our field along with a summary of our fiscal year 2023 financial performance and a preview of our budget for fiscal year 2024, and as a reminder, our year at the Foundation Fighting Blindness ends in June and begins the new year in July. So we're really just a month and some change into fiscal 2024 already, so it'll be a good time for Peter to review the year behind us and also preview the year ahead.

Next, we're going to have Todd Durham, who's our Senior Vice President of Clinical and Outcomes Research, and Dr. Claire Gelfman, who's our Chief Scientific Officer. Together, they're going to provide updates on recent research and development news, clinical trial related developments and other things from that perspective, and then I'll return to the call a little bit later to discuss several key strategic initiatives, and it'll be my pleasure at that time to introduce two really special and important people within the Foundation Fighting Blindness community who are going to highlight the success of our recent Victory for Vision campaign.

First will be Judy Taylor, who is our Senior Vice President and Chief Development Officer here at the Foundation. She's responsible for coordinating all of our many fundraising efforts across the globe. And then Dr. Marsha Link, who is not only a high profile ophthalmic industry veteran, but she's also a passionate board director here with the Foundation and one of our co-chairs for the Victory for Vision campaign.

Following all of these formal remarks like we always do, we'll open the call for your questions. In addition to this team that's on the call right now, we'll be joined by Dr. Amy Laster for the Q&A session. Amy is our Senior Vice President of

Science Strategy and Awards and she'll be joining us for the Q&A session today. With that, I'd like to turn the call over to Chris, our Vice President of Marketing and Communications, Chris Adams.

Chris Adams, Vice President, Marketing & Communications:

Thank you, Jason. Back in July, not only did we start our new fiscal year, but our teams have hit the ground running. There are many initiatives in progress, and I will highlight several of them on today's call. To drive awareness for the Foundation and the work we do we are in development of a new public service announcement, or PSA, campaign, which is planned to launch in October during Blindness Awareness Month. For the past two PSA campaigns, we've received over 1.4 billion impressions, which equates to more than $31 million in free media advertising.

The message for this year's campaign focuses on breaking the stereotype of what being blind or having low vision looks like. The campaign will feature a variety of individuals from our community and will consist of television and radio commercials along with print and digital advertising. Also, later this year, we will complement the PSA campaign with targeted advertising efforts driving awareness and engagement for the Chapter Network.

This fall brings many events to our community, including chapter activities, vision walks, golf events, and dinners being held throughout the United States. One event I would like to highlight from last month is the 23rd annual Microsoft Scramble for Sight presented by RE/MAX that was held at the Sanctuary Golf Course in Colorado. This event exceeded expectations showcasing the power of Unity by raising over $355,000 for our mission. The event was led by an incredible volunteer committee featuring co-chairs Scott Burt, who is a Foundation Fighting Blindness board member, and Sherri Kroonenberg, one of our national trustees. There were more than 120 golfers and 200 attendees who united to raise awareness and funds during the event.

To see what events are planned in your community, please visit the Foundation website at fightingblindness.org and select the Foundation events tab in the upper right hand corner. From there, select View All Events to see everything

going on. If you have a chapter in your area, you can also see the upcoming activities on the specific chapter page. To find out more about our chapters, please visit fightingblindness.org/chapters.

Lastly, we are excited to share that the Foundation's National Conference Visions 2024 will be held June 21st and 22nd at the Chicago Marriott Downtown Magnificent Mile. Our conference will feature sessions on the latest research advancements, practical adapting and thriving, and an opportunity to connect with others from the blind and low vision community. You can find more information about the Vision's 2024 conference by visiting our website. Be sure to check back frequently as more details will be added. I'm now pleased to turn the program over to Peter Ginsberg, our Chief Operating Officer, Peter.

Peter Ginsberg, Chief Operating Officer:

Thanks, Chris. On today's call, I'm pleased to walk through a few noteworthy developments along with an update on our financials. Earlier this month, the FDA approved IZERVAY for the treatment of geographic atrophy associated with dry AMD. IZERVAY was developed by Iveric Bio, which was recently acquired by Astellas. You may know that dry AMD patients now have two FDA approved drugs, IZERVAY and Apellis' SYFOVRE, up from zero at the beginning of this year. Claire will provide more details in her remarks, but we at the Foundation are very excited that there are now two new GA treatments and we greatly appreciate the support of Astellas, Iveric and Apellis as outreach sponsors to the Foundation.

Also in further good news, earlier this month, ProQR announced the sale of its two ophthalmic programs to Laboratoires Théa, which is the leading independent eyecare group in Europe with 1,700 employees and roughly a billion dollars in sales last year. The R&D Fund co-funded the development of one of these two therapeutics targeting Usher syndrome type 2A, and with expertise in the research development and commercialization of eyecare products, Théa is very well positioned to continue the development of these two programs for patients with rare genetic eye diseases.

Switching gears, I'd like to provide a brief summary of our fiscal year 2023 financials and our budget outlook for fiscal 2024. The Foundation operates on a

fiscal year that runs from July to June, so we just completed our fiscal 2023 on June 30th. I am pleased to report that our preliminary unaudited financial results for 2023 indicate that we exceeded our budgeted $13 million net fundraising surplus for the year. Overall, again, preliminarily, we spent $27 million on research and education in fiscal 2023. I want to repeat that because it's a very important statement. Preliminarily, we spent $27 million on research and education in fiscal 2023.

For fiscal 2024, which is the current fiscal year that we just started, we're targeting $34.5 million in unrestricted revenue against $21.1 million in operating expenses for $13.4 million net fundraising surplus. That surplus of more than $13 million that we're budgeting for fiscal 2024 will go to support new research funding.

As we wrap up the last fiscal year and plan for 2024, we have our regular annual audit ongoing, and we expect that our audited financial statements will be available this fall on the Foundation's website in the About Us section under Financial Reporting.

Now, timely reporting of our financial results is part of our commitment to being a model nonprofit organization. As Jason explained on one of our previous Insights Forums, we want to be a leader, not just in our field, but across the spectrum of nonprofits in terms of how organizations operate and impact their respective missions. With that goal in mind, we're pleased to report that we continue to make progress as measured by outside rating agencies that assess nonprofit effectiveness. Our Charity Navigator rating has increased to the highest rating of four stars and our Charity Watch rating improved to B+. We're quite encouraged by these increases, but you can count on us to continue to strive for the highest ratings possible.

With those updates, I'm now pleased to hand the program over to Todd Durham, our Senior Vice President of Clinical and Outcomes Research. Todd.

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

Thank you, Peter. I'd like to share with you some of the recent progress and activities that we're working on to advance clinical and outcomes research

activities. In order to find treatments and cures, it's critical that we understand the natural progression of the diseases we're targeting and in dealing with inherited retinal diseases, there are nearly 300 different conditions related to genetic variations. Natural history studies help us to evaluate how a disease progresses, estimate the rate of disease progression, and how variable that progression is among groups of effective people. Our ultimate goal is to boost and accelerate the development of therapies among both academic researchers and industry.

During our Retinal Cell and Gene Therapy Innovation Summit in April, there were a number of presentations about the many natural history studies that the Foundation has helped to design and fund. This included an update on our Uni-Rare Study, which is the first of its kind.

We've talked a bit about this study on previous Insights Forums, but as a reminder, it's a new type of natural history study for people with one of the more than 300 genes associated with inherited retinal diseases. Since the more common IRD causing genes have been the subject of other clinical trials, the Uni-Rare Study focuses on the less common or more rare forms of IRD genes. This study gives us a great opportunity to understand and learn more about those rare genes that would otherwise not be captured in a natural history study that was focused on a single gene.

The Uni-Rare Study is well underway. We began enrolling patients in May of this year at the first of our 30 planned clinical research sites. We're working to have all these sites open to enroll participants and we will provide updates in future calls.

If you'd like to learn more about this study, including the list of participating research sites, please go to www.clinical trials.gov and type in the search bar Uni-Rare. And of course, if you need help or have any specific questions about Uni-Rare, you can always email us at info@fightingblindness.org and we can help direct you to a research site that is most convenient for you.

We also have positive news to report on another of our sponsored natural history studies. Later this month, we expect to enroll the first of 45 planned participants in the GYROS Natural History study. GYROS is a four-year study of people affected

with gyrate atrophy, which is associated with mutations in the OAT gene. The mutations in OAT cause elevated plasma levels of the amino acid ornithine and the ornithine accumulates and causes unwanted effects in the eye, including the retina. Gyrate atrophy typically presents in childhood with night blindness and progresses with the loss of peripheral vision and it may eventually lead to the loss of central vision. The GYROS study will inform the design of clinical trials of a gene therapy being developed by researchers at Johns Hopkins University.

GYROS is being funded through a collaborative effort involving three groups. The Foundation is providing $1.8 million in funding. The study is also supported by the Food and Drug Administration, or FDA, of the U.S. Department of Health and Human Services as part of a financial assistance award totaling $1.6 million. And finally, a nonprofit research and advocacy organization called Conquering Gyrate Atrophy is contributing $100,000 to GYROS.

Another area of focus for our team is working collaboratively with regulators to identify meaningful clinical trial designs and endpoints. And to address this, we have a working group called REDI, which stands for Regulatory Endpoints and Clinical Trial Design for IRDs.

We held our most recent REDI working group meeting with the U.S. FDA in June. We presented data on functional and structural endpoints from one of our recently completed natural history studies, RUSH2A. This four-year study, which completed enrollment in 2019, studied retinal degeneration caused by mutations in the RUSH2A gene, which is a leading cause of Usher syndrome type 2A and autosomal recessive retinitis pigmentosa. We enrolled approximately 125 patients at 20 sites in the U.S., Canada, and Europe. The presentation of the RUSH2A data helped facilitate a valuable discussion with regulators about potential new metrics or endpoints that can be used in clinical trials to show that an investigational treatment offers some benefit to study participants.

The evaluation of new endpoints considers a number of factors including how practical the measurement is, how reliable or repeatable it is, how much it changes over time, how variable or noisy it is, and how it relates to outcomes that are meaningful to patients. We're taking this feedback and sharing it with the broader research community to help inform future trial design.

We are encouraged by the continued progress in learning more about IRD progression through our natural history studies and believe these initiatives will have numerous benefits for our community. And with that summary, I'd like to hand the program over to Dr. Claire Gelfman, our Chief Scientific Officer. Claire.

Dr. Claire Gelfman, Chief Scientific Officer:

Thank you so much, Todd. Good morning everyone. On today's call, I'm going to provide an update on our current research funding. I'll summarize new grants awarded and highlight recent developments in treatment of age-related macular degeneration, also known as AMD.

The Foundation is able to fund a very diverse portfolio of emerging therapy 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.

We're currently funding 83 active grants, which are being conducted by more than 100 research investigators at 68 institutions, hospitals and universities. In addition to funding researchers within the United States, Foundation funding extends internationally laboratories in Australia, Belgium, Brazil, Canada, England, Finland, France, Germany, Israel, Italy, Mexico, Netherlands, Poland, Spain, and Switzerland. As part of our 2023 funding, we recently announced the recipients for individual investigator research awards. Based on a rigorous review by our Scientific Advisory Board, we are funding nine new awards for fiscal year 2023.

These awards are going to researchers at the University of Alabama Birmingham, Ghent University in Germany, Michigan State University, Columbia University, University of Delaware, Center for Genomic Regulation in Spain, University of Washington, Baylor College of Medicine, and Duke University. And of these award winners, six investigators are new to the Foundation, meaning that this is their first time receiving an award from us.

Researchers at these institutions are working on a wide range of cutting edge approaches to understanding and creating inherited retinal disease. This includes research on PRPH2, ABCA4, CRX-associated retinal degeneration, as well as research to identify novel treatment targets as well as tools to better image

children with inherited retinal diseases. In addition, we are funding two program project awards. One award was granted to Hadassah Hebrew University, which is investigating the use of RNA editing technology to correct specific retinal disease-causing mutations. The second award went to Fordham University with his working on the disease pathogenesis and treatment solutions for vision loss due to retinitis pigmentosa associated with mutations in the MERTK gene.

For our portfolio of career development awards, we announced three early career recipients, Dr. Robert Hyde at the University of Illinois Chicago, Dr. Debarshi Mustafi at the University of Washington, and Dr. Katherine Uyhazi at the University of Pennsylvania.

In addition, earlier this year, we announced the 2023 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.

As we look forward, our Board of Directors approved our fiscal year 2024 science budget for new research funding of $17 million as part of our five-year strategic plan.

As I wrap up, I'd like to highlight a few developments in the clinical research landscape. As Peter mentioned earlier this month, Astellas Pharma announced the FDA approval of IZERVAY for the treatment of geographic atrophy, secondary to advanced dry AMD. IZERVAY was developed by Iveric Bio, which was acquired by Astellas in July 2023. Astellas expects IZERVAY to be available in the U.S. very soon.

More than 150 million people worldwide have AMD, including 10 million in the U.S. Of that total, approximately 1.5 million people in the U.S. have geographic atrophy, which is a more advanced condition, the leading cause of devastating central vision loss in people 55 years of age and older. Vision loss caused by geographic atrophy is from the toxic deposits known as drusen that accumulate underneath the retina. The degeneration of those neighboring retinal pigment epithelial cells ultimately leads to loss of photoreceptors and central vision loss.

IZERVAY, which is administered through an intravitreal injection in a doctor's office, is designed to work by inhibiting a protein known as C5, which is part of the complement system. Complement is part of the innate immune system and therefore plays a very important role in fighting off viruses, bacteria, and other pathogens. Researchers believe it's an overactive complement system that is a key culprit in the development of AMD.

IZERVAY and SYFOVRE, which was the first product approved for GA last April, work by inhibiting the complement pathway. We are so very encouraged that we have two approved products for geographic atrophy. However, there remains a high unmet medical need globally for a range of treatment options for patients who may respond differently depending upon their disease state. To that end, there are more than 10 other companies advancing potential treatments for dry AMD. More information on these programs can be found in the clinical trial pipeline section of the Foundation's website.

It's truly exciting to have multiple therapeutics in development as well as two new FDA approved treatments to help people with dry AMD maintain independence and quality of life. This is really what motivates our team to ensure that we can continue to fund innovation, new developments and clinical progress. Research awards we are making now will fuel our future achievements and ability to ultimately help everyone diagnosed with those inherited retinal diseases and dry AMD, regardless of their gene mutation and degree of vision loss. With that, 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 again, thank you to everyone really for joining us today. Peter mentioned it a little bit earlier in his section, I know I've mentioned it on prior Insights Forum calls that we really do aspire to be a model non-for-profit organization and a global leader in how we go about advancing our mission. I really do believe that frequent and transparent communications with you, the people in our community are really an important way in which we're doing that and really an important way in how we go about living our mission. So I do want to thank each of you. There's typically thousands of folks who are on these calls - they're watching on Facebook, streaming it, watching live on Zoom or watching it

on replay. At this moment there are several dozen questions that have already been chatted in, and really that engagement with our community is why we do this, and I really do appreciate everyone carving out time to join us today.

I do have several strategic developments that I want to highlight today, and I want to start my section today with a very important announcement. We announced earlier this month that David Brint, who has served as Board Chair for the Foundation since 2016, has made the very difficult decision to retire as chair of the Foundation Fighting Blindness effective next June, that's June 30th, 2024.

It's really hard to put into words the tremendous impact that David has had in serving the Foundation and really serving this mission as Chair. His contributions began well before his time as Chair, and I'm certain that his contributions to our mission will continue well after his time as Chair. His impact has really been significant. To facilitate a smooth transition, a special committee was formed to identify a successor for David, and we are thrilled to announce that Karen Petrou has been elected as new board Chair effective July 1st, 2024, so that'll be next July.

As part of this transition, Karen has been named to a newly formed position within the board of directors entitled the Executive Vice Chair, and in this role Karen will work really closely with David, with myself, and of course the full board trustees and you in our community in preparation to assume her very important role as Chair next July. For those of you who don't know Karen, she's a fantastic person and I know she's anxious to meet as many of you as she possibly can. Her background in her connection to our mission is a very personal one. Karen was diagnosed with retinitis pigmentosa at the age of 18. She has served on our board at the Foundation since 2006. She will become the third Chair of the Foundation in our 52-year history, so that's something, and she'll be the first female Chair in our Foundation's history.

Professionally, she's the co-founder and managing partner of Federal Financial Analytics, which is a company that provides analytical and advisory services in a variety of different ways, including public policy, legislative, regulatory issues, and really a world-class consulting firm as it relates to financial services and financial

policy, doing business not only here in the United States, but really all over the world.

She's a strong and vocal advocate for our mission. She's a strategic leader. In all ways, she is truly the perfect successor to David's many years of service. And as part of our upcoming Visions 2024 conference that Chris mentioned earlier in today's call, we will celebrate David's impact on the Foundation and the organization and our mission as Chair and officially welcome Karen as our incoming Chair for the Foundation's board.

Our board members really are critical in helping support the Foundation in so many ways. One of the great examples of this relates to a topic we're going to have on today's call, which is our multi-year capital campaign that we've been talking about on these calls for the last year, the Victory for Vision campaign. Many of our board members have served in various volunteer roles in helping to lead the effort with Victory for Vision, and we were blessed by having three co-chairs of the campaign that are all board members, and that includes David Brint, Robert Heidenberg, and our special guest speaker for today, Dr. Marsha Link.

A major strategic initiative like the Victory for Vision campaign is really necessary to fund the research on future treatments and cures for those impacted by inherited retinal disease in dry age-related macular degeneration. The Victory for Vision campaign was launched back in 2021, and it really was an urgent call to action for our community to provide specific, attainable goals for our staff, our board, our leadership, and all of our volunteer community to rally around and we set the bar high. When the campaign was launched, you may recall, we set the bar for an incremental $50 million to be raised over a five-year period on top of our traditional annual fundraising goals. The response was overwhelming.

Based on the tremendous effort of our campaign volunteers, our leadership team, our staff, and many of you on this call today who contributed to the campaign with time or treasure, we have surpassed the $50 million goal quickly. And then you may recall we raised the bar, we took the goal from $50 million because we had already met that bar. We raised it to $75 million. I'm thrilled to announce today that we've also surpassed that goal and we continue to march forward to raise as much as we possibly can to advance the work of our mission.

So if you fast-forward to where we are today, it's my pleasure to introduce two key members of this campaign so they can share a little bit about our milestones, and importantly a vision of where we're going next. I'm thrilled to have Judy Taylor and Dr. Marsha Link joining us on today's call.

I'm going to first introduce Judy and her role as the Foundation's Senior Vice President and Chief Development Officer. Judy is responsible for the strategic direction, planning and oversight of our major gifts functions, our legacy giving functions, all of our chapter engagement that happens across the country and has been our staff lead on this critical initiative, the Victory for Vision campaign.

Judy's worked at the Foundation for nearly 10 years. She'll be the first to tell you she's worked at the Foundation in two different stints. She left and then loved it so much that she came back. In her last four years here in her current role, she's made tremendous impact. She has 30 years of experience developing innovative fundraising programs and engaging donor audiences and really is an invaluable member of our team. And I'm also especially pleased to welcome Dr. Marsha Link. So after Judy, we're going to hear from Dr. Link. I've had the pleasure of knowing Marsha for so many years. She's a leader in the ophthalmology community. Many in the ophthalmology community know her, know her energy and her passion, and she's one of the perfect people to lead this campaign. We're going to hear from both of them now and I'm going to start by turning the call over to Judy.

Judy Taylor, Senior Vice President, Chief Development Officer:

Thank you, Jason. Good morning everyone. As Jason said, my name is Judy. I'm going to take a few minutes to talk about the Victory for Vision campaign and you'll continue to hear just how incredible and an impact the campaign has had over the last several years. The timeline for the campaign is January 2021 through December 2025. Really the success of any fundraising campaign begins with the outstanding and committed leadership. The campaign committee and the staff leaders for Victory for Vision campaign were dedicated and determined to meet the campaign goal of $50 million, which you already know, we increased that goal to $75 million.

Our Board Chair, David Brint, and our board members, Robert Heidenberg and Marsha Link stepped up to lead this really important effort. Serving on the campaign executive committee, these three leaders were thought partners for campaign goal setting, for the strategy, campaign ambassadors, solicitors, and worked to keep the committee engaged and motivated. And boy did they do just that.

I'm going to talk just for a minute about the campaign structure. It really called for five different divisions. We have so much appreciation and are grateful to the following leaders and committee members that were over these divisions.

The Board of Directors and National Trustee Division was led by Jordan Bergstein and Jason Morris. Major gifts, or transformational giving, was led by Eddie Russnow and Scott Selby with Michelle Dudley and David Nixon serving on the committee. Our international division was led by Laura Fietta. Our legacy division was led by Dan Day and Davida Luehrs with Joel Davis, Sherri Kroonenberg and Martha Steele serving on the Legacy Committee. Our next generation or broad base, is led by Christine Exley and Drew O'Brien, and that division includes multiple tactics through chapters raising our sites, our DIY platform, direct marketing and corporate partnerships. A lot in that one division.

These various divisions were executed in phases with some overlapping and some ending sooner than others. The campaign moved fast like Jason mentioned, and faster than we even anticipated, and the revenue goal was increased from $50 million to $75 million. And what that looks like is that $30 million was transformational giving, our major donors. $30 million for legacy, and that was our documented commitments. $5 million is engagement, that broad base for really our grassroots efforts. And then we had a really special $10 million bonus gift from a very generous donor, which really helped us and allowed us to surpass that $75 million goal. We are going to keep the campaign going to maximize its overall impact. So more to come on that, but it really was impactful and we can't say thank you enough for all the support that this has had and the impact on the research.

I'm very excited now for you to meet our next guest, our campaign co-chair, Dr. Marsha Link. She is here to share her perspective on the importance of this

initiative. Marsha wears many hats - an executive, human resources professional, educator, coach, wife, mother and grandmother. She has been involved in the ophthalmology sector for many years. Marsha has served in a variety of nonprofit organizations and she's especially eager to find treatments and cures for retinal diseases since she has a family member with retinitis pigmentosa. She's one of my favorite people and I think you'll see why as you hear from her today. I'd like to hand it over to Marsha to share more details. Marsha.

Marsha Link, Board Director and Co-Chair, Victory for Vision Campaign:

Thank you, Judy. I'm happy to be here with all of you. I just wanted mention that the purpose of the campaign, as you've heard, is to continue to accelerate the best research in all stages of development and to raise funds above and beyond what we saw from our traditional sources of revenue. And in order to build the Victory for Vision campaign, we created this global multi-tiered giving strategy, which Judy has described, and we have galvanized the Foundation's donor journey to broaden the base of supports to fund the life changing research.

And with the $75 million that we've raised over three years now, we can award more than 50 grants and expand our national history study, as Todd has already mentioned. We can begin to make the theme of this campaign, which was Together We Are Winning, a reality. With the dollars we have raised, these are a few things we can do. We can invest in a dozen early career clinicians and researchers, fund three team-based collaborative projects, support more than two dozen preclinical research projects, invest in half a dozen core resources that support translational research like the large animal models of IRDs, accelerate a dozen translational research programs that gets us closer to the clinic, which we all want those treatments, and expand the Uni-Rare study to enroll more than 1,000 people with IRDs.

Now, that's really exciting news, but we want to keep up this momentum. And in fact, the Foundation is now $100 million away from achieving a $1 billion lifetime funding. Over the next few months, we will share more with you about plans to cross that billion dollar threshold, so stay tuned for that. I'm grateful and honored to be part of the Foundation's missions and efforts to make a difference in our community and our own family. Like many of you, I have a loved one with an IRD,

and with your help and the help of many others, we are confident that we can win. Thank you for your time and your commitment, and now I'll hand the meeting back to Jason.

Jason Menzo, Chief Executive Officer:

Thank you so much, Marsha. It's great to hear your voice. I know our constituents all over the globe who are tuning in today can feel your passion and your energy. And Judy, your strategic direction in laying out not only how we've gotten to where we are now, but most importantly where we're going as we continue to capitalize on the success of the Victory for Vision campaign. My friends on the staff and certainly on the board don't love it when I make these insinuations, but we started with a goal of $50 million, we surpassed it, we then moved the bar to $75 million, we're there. How high can we go? Because every dollar that we raise is again, more impact in advancing why we're all here, which is treatments and cures for inherited retinal diseases and dry age-related macular degeneration.

I do want to encourage everyone on the call today to check out the website fightingblindness.org. Learn more about the campaign. If you haven't made a contribution to the campaign yet, there's great opportunity to still make a difference. Every dollar that we raise through the campaign or through any of our other many initiatives really does make a difference.

A key part of our strategy as we've discussed at length today is reaching out to constituents, not just here in the United States, but really across the globe. We see in the chat every time we do one of these Insights Forums ... this is live by the way. We don't prerecord these. This is live right now unless you're watching two weeks from now on a replay on Facebook. But in real time, we have people chatting in from all over the world, from countries that I'm familiar with, like the handful of folks that have chatted in saying, "Hi from the UK," but also from countries I'm not as familiar with.

It's just a reminder that while the community is global in nature, the impact that we all make together is very personal. We're all in this journey and on this journey for our mission because of personal reasons, but it does take our entire global community working together to help advance the milestones to meet our

mission. As Claire mentioned, we're currently funding a numerous research programs and projects and companies advancing our mission really across the globe. And as part of our extended outreach, the Foundation will be hosting our first international webinar highlighting the global research community and projects that we fund from all over the world. This is coming up in just a few weeks. I would really encourage everyone who's on the call today from outside the US or even from within the US, to call in and join us for this event. So please mark your calendars to participate in this webinar. It's going to be held on Wednesday, September 27th, 2023 at 5:00 PM UTC or 1:00 PM Eastern here in the United States.

If we step back for a moment, it's truly humbling and inspiring to think about the impact that we can make and continue to make if we get to the milestone of surpassing a billion dollars raised to go towards our mission. We really can win this fight and it's going to take all of us as a community working together to continue to push and keep the momentum going that we're experiencing right now.

It is that time of the call, it is here on the eastern coast of the United States 11:44. So we have 15 or 16 minutes for Q&A, which is great. We have dozens of questions already in. I'm going to turn it over to Amanda to remind us how to ask your questions and then we'll jump right into it.

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 functions, both of which are at the bottom of your Zoom screen and make sure you include your names 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 or so. Back to you, Jason.

Jason Menzo, Chief Executive Officer:

Very good. Judy, I'm going to start with you. You mentioned our international goal associated with the campaign. You've talked at length today about our extending the reach beyond the U.S. borders. Also, we just said this a minute ago, we're

announcing our international webinar coming up in September. Can you talk just a little bit about what those strategies are? What could folks learn on that webinar? Why are we moving in this sort of direction? What should folks be aware of?

Judy Taylor, Senior Vice President, Chief Development Officer:

Thanks, Jason. This is Judy. I think a forum like the international webinar really allows us to show our constituents the global impact that we're making. One of the things that we will include on the webinar is the launch of our development strategy, which is coming out of the success of our Victory for Vision campaign. We've done a few outreach events. We did a CEO Roundtable in London with so much success really showing us that there's a need. We were able to establish a number of partnerships during that CEO Roundtable discussion. We've hosted some receptions in the UK, so we're seeing the need and also the impact overall with our science.

Joining that webinar, we will have researchers from Spain, Italy, London, and Israel giving us some insights of the research that we fund globally. I think we're really going to see some momentum as this becomes a focus for us and one of our priorities, we're going to see this grow and be able to report out our findings and our successes and our challenges on the Insights Forum calls coming up.

Jason Menzo, Chief Executive Officer:

Thank you, Judy. And I should have mentioned for my colleagues, so Todd, Claire, Peter, Chris, Amy, please turn your cameras on, come off mute because I'm going to be sending questions all of your ways here in just a minute. Claire, I'm going to come to you next. We've talked quite a bit in prior Insights Forums about the optogenetics approach, and there were some questions about what is the latest in optogenetics, what type of vision might one expect? And again, that's speculative because many of these are not yet in the clinic, but maybe you could frame a little bit about the expectations, the strategy, and what is the latest in the optogenetics field.

Dr. Claire Gelfman, Chief Scientific Officer:

Thanks, Jason. This is Claire Gelfman. So optogenetics is really all about providing vision to an individual who has really late stage photoreceptor degeneration, meaning that there's severe vision loss where you really don't have the remaining photoreceptors needed to respond to light so that you can see what's in front of you. And one of the unique features of optogenetics is that it's getting that light sensing capability to a part of the retina that's not degenerating. It's important to manage expectations about what that might look like in an individual who is given this type of treatment.

As I mentioned before, it's really for individuals who have late-stage degeneration because the amount of improvement in vision is going to be small. If you have a certain amount of functional vision, you may not notice a difference. However, the individuals who are enrolled in the clinical trials for optogenetics have either no light perception or are totally blind, and the small increment in vision improvement is a huge quality of life improvement.

People have noted things like shapes and crosswalks outside, things that can really make a difference if your starting place was really no functional vision. I really want to manage that expectation, and that's in contrast to some of the other therapies we talk about that are for individuals who still have some remaining functional vision. The other important thing to note about optogenetics is that the treatment is what we call gene agnostic, in that it's not one that is specifically targeted towards one genetic mutation or another, but rather for an individual who's been diagnosed with retinitis pigmentosa who could receive this therapy to then provide a new avenue to respond to light and to see that small, albeit incremental, vision improvement that could be beneficial to someone who is experiencing late stage photoreceptor degeneration.

Jason Menzo, Chief Executive Officer:

Thank you so much, Claire. I'm going to shift and actually in contrast to gene agnostic approaches, which we just described with optogenetics, every time we have an Insights Forum, there are dozens of questions that are chatted in about specific genes and some of them we'll definitely address here today. In particular, we'll get to a big contingent of our community, which is Stargardt. So as someone

just chatted in, I missed the update on Stargardt. We haven't gotten to it yet, so don't worry. We're going to get to that in a few minutes.

But for the specific questions about particular genes, we may not get to all of them here on the call. As Chris has put in the chat and we've mentioned several times, we do follow up with every single question that is chatted in, and so be on the lookout for that. Todd, let's shift to MRTR. Always a lot of questions. Today's no exception. How do I go about getting tested? When do I get retested if it was inconclusive or there was no pathogenic variant found? How do I get my results? How is a genetic counselor involved? Maybe you could talk just a little bit about that process.

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

Hello everyone. This is Todd Durham again. We operate a study called My Retina Tracker Registry that helps us understand the genetic causes of inherited retinal diseases. And one of the ways that we make this research work is we offer a no cost genetic testing and counseling session to individuals with inherited retinal diseases. Through a program with our partner, Blueprint Genetics, you can participate in our study and gain access to this panel test, which has over 300 genes on it, which means it'll be fairly comprehensive for individuals with inherited retinal diseases.

If your provider in your state is legally allowed to order a diagnostic test, then they are allowed to order this test through our program. So your provider could be a low vision specialist, an ophthalmologist, or an optometrist if they're allowed in the state to order the test. So you can reference our website, My Retina Tracker Registry, or your provider can go to Blueprint Genetics and look up the My Retina Tracker program and they can learn more about this test.

But one of the issues I wanted to raise is once you have ordered the test and you access it, you will receive a genetic counseling session either with your provider or a genetic counselor at their site or a genetic counselor associated with Informed DNA, and just can't emphasize enough how important this genetic counseling session is. Sometimes the results are fairly straightforward in that you get a clear cut picture of what's causing you an inherited retinal disease. Sometimes the

answer is not very straightforward, that means it's perhaps inconclusive. Maybe there's a sense there that they've identified the causative gene, but they can't be totally sure that they've got enough evidence.

This is really based on rigorous evidence base of accumulated cases. That's how this works. So they really need to make sure they have enough data to support the diagnosis. Sometimes there are cases, and I see someone issued a question about this, sometimes there's cases where they couldn't identify a gene at all, and that does happen sometimes, and there are research endeavors underway to try to find in those cases are there new genes that we have not discovered? Or perhaps there's more complicated genetic relationships where perhaps there's one gene over here that causes the condition, but you need to turn on, maybe there's some other modifier gene that explains what's going on that we don't currently test for.

Genetics can be fairly complex. So the genetic counselor can help you interpret those results both in terms of what does it mean for you in your daily life and your planning, but also what are your next steps? They would be the best resource for you to understand when might I go retest again. And when to retest again, really depends on what's the nature of the panel that was used to test. The Blueprint panel is quite comprehensive, but even then we come back with some results that are negative. So consult your genetic counselor on that particular question is the best advice I can give.

Jason Menzo, Chief Executive Officer:

Thank you so much, Todd. I do want to mention a few minutes ago someone had raised their hand from a 508 area code. I see now they've taken their hand down, but whoever that individual is, if you would like to come off mute and ask your question live, just go right ahead and raise your hand again and Chris will be ready to unmute you. But in the meantime, Amy, I want to come to you.

Many times, it does happen that someone goes through My Retina Tracker and they receive a genetic test and then the results come back that there is either no pathogenic variant found or maybe a variant of unknown significance, and they have a conversation with a genetic counselor and at some point they may get

retested, but they may be wondering what efforts do we have at the Foundation to identify additional genes that are not currently identified? And we call that the elusive gene type of research, that project. Maybe you could talk a little bit about what that is.

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

Thank you, Jason. So the Foundation, we do support several research projects through what is called our Elusive Genes Initiative. And again, this is like unrecognized genetic causes of IRDs, the specific genetic cause of the disease, it essentially remains elusive. This is true for nearly half of the known IRD populations, and oftentimes it is autosomal recessive diseases that's most challenging.

Now while elusive genes are thought of as genes that are not previously identified, so something that we haven't even found yet being associated with IRDs, research that's funded by Elusive Genes Initiatives is revealing that some of these elusive causes really can be attributed to some pathogenic structural changes like rearrangements within the genes or duplications within parts of the genes, or maybe things are kind of swapped in located and genes that are already known to cause IRDs and sometimes it's just some technical weaknesses of the existing technology that we do use in traditional genetic analysis.

I just highlights the importance of really continuing to adapt the genetic testing platform as new mechanisms of diseases are identified. And so through our Elusive Genes Initiatives, researchers are able to continue to expand the gene panel that Dr. Durham just mentioned for individuals whose genes mutation has not been identified. We are constantly seeing that there are new elusive genes that we know. And then for the populations in which ... and sometimes it's very specific like in Stargardt, if you have ABCA4, that comes back as a variant of unknown significance, that there are new tools to actually go in and say, well, let's check and see is this one of those kind of structural rearrangements that may be causing the disease.

Jason Menzo, Chief Executive Officer:

Thank you, Amy. Appreciate that. We do have a couple more questions that we're absolutely going to try to get to today. There's many, many more that we will not get to on the webinar, but we will follow up with you individually. But I do want to take an opportunity to go to a couple of these folks that have their hands raised. So Chris, if you don't mind, let's try to unmute the line of the individual with the 508 area code.

Jane Perry, Participant

Yes, hello. Good morning or good afternoon. This is Jane Perry from Falmouth, Massachusetts on Cape Cod and I've been a member of FFB since 1988. Thank you very much for this call. Having retinitis pigmentosa has changed my life in a very positive way and I was very involved with the Massachusetts affiliate in the 1990s. I have a quick comment and then a question. I am also a member of the American Council for the Blind, and this July at our national conference in Schaumburg, Illinois, you made my dream come true because it was the first time that they came to an ACB conference. They gave a presentation, I forget the gentleman's name, on Thursday at the last general session, and he was there at the information booth all week. So thank you, thank you, thank you.

Here's my twofold question. I have Bardet-Biedl syndrome, gene one, I have a second cousin, which is very rare, I guess that has another syndrome of RP. She has Usher's type 2B. So my two questions are I have spoken at several conferences before and I'd like to get back involved with the conferences to participate on the rare syndrome track, who do I contact, number one. And number two, what is happening with any trials for Bardet-Biedl syndrome? At Marshfield Clinic, there are 700 plus people involved in the CRIBBS registry and also it's great to hear about Usher's type 2A. I hope type 2B will come along. Thank you very much for allowing me to speak and I will be back in contact with Martha Steele and get more involved with the Boston chapter. Thank you again.

Jason Menzo, Chief Executive Officer:

Thank you very much for the comments and for the question. We love Martha Steele, so I know she'll be thrilled to talk to you. What we can do, so to answer your question, who to get in contact with, and this is not just for this question,

but really anyone on this call that has a question that they've asked today or something they think of down the road, we have a general way to get in touch with us that we can then triage to the right person. That is just by sending an email to info@fightingblindness.org. We will then ensure that the right person can get back in touch with you. We will, in that same way, circle back with you and we can have a discussion about what's happening with Bardet-Biedl syndrome specifically and also Usher's 2A because there's a lot of progress happening specifically with Usher's 2A.

But my message here is that anyone who has a question that they didn't think of right now or needs to get in touch with the Foundation for any reason can use the info@fightingblindness.org mailbox. I'm going to try to see if we can tackle a couple more questions. There were several about the current landscape of Stargardt, and so Amy, I'm going to ask if you can speak a little bit about both the preclinical and certainly in the clinical realm. Maybe you could speak a little bit to that. And then after Amy, Claire, I'm going to come to you to answer some questions about nutraceuticals and some news that has been sort of in the lay press around turmeric and cumin and some work that we're doing with UCSD in that regard. So we'll start with you, Amy, on Stargardt. And then we'll come to you, Claire.

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

Thank you, Jason. Again, this is Amy. There are several pharma companies, including Alkeus, Belite, as well as the National Eye Institute that are testing some small molecules or drugs in clinical trials to address Stargardt's. Additionally, there's a clinical trial using an optogenetic strategy that Dr. Gelfman described earlier are currently underway, sponsored by Nanoscope. Really, for more details about each of these, as well as some others that I didn't name specifically, please visit the Foundation's website, Foundation Fighting Blindness under Clinical Trial Pipeline, or you can go to clinicaltrial.gov.

Now in addition, the Foundation continues to support research laboratories and currently several that are evaluating a lot of the basic research that is necessary to advance these clinical trials or to move new therapies into clinical trials, as well as looking at the genetics of Stargardt for ABCA4, developing animal models as

well as cell-based models to understand more about Stargardt's disease as well as using these to test potential treatments and develop new treatment therapies for Stargardt's disease.

Jason Menzo, Chief Executive Officer:

Thank you, Amy. Claire, actually, I'm going to see if we could do a one two punch and send you two questions in one sitting here. First, can you discuss a little bit about the question or the news around nutraceuticals in the space, in particular, turmeric and cumin and some work that we're doing at UCSD. And then separate from that, the jCyte trial, which is very different in nature, but there have been several questions about the current status of the jCyte trial, what is known publicly that we can share with the audience today?

Dr. Claire Gelfman, Chief Scientific Officer:

Thanks, Jason. This is Claire. I'll start with the second question with jCyte. As you know, jCyte as a cell-based therapy to treat individuals with retinitis pigmentosa, it's all about delivering cells that will make proteins needed for the existing cells to reinvigorate them. We get a lot of questions about jCyte in these forums, and what we have learned from our colleagues at jCyte is they do have plans to initiate their Phase 3 trial in the first half of 2024, so the first half of next year, they plan to initiate this Phase 3 trial. In terms of the actual enrollment, when they post the initiation on their website, they will let us know so that we can also communicate that through our site. The next step being enrollment. So stay tuned for the initiation the first half of next year.

Regarding nutraceuticals, there has been a lot of attention regarding curcumin and turmeric, mostly anecdotal, but very compelling for individuals with retinitis pigmentosa. Our colleagues at the University of California San Diego published some preclinical studies and mouse models that showed some real compelling data, and I think with nutraceuticals, it's really important that before anyone reads information and before they start taking anything, it’s really important to speak with your ophthalmologist and make sure that it's the right thing to do with respect to your specific situation. Again, there's a lot of compelling evidence out there, but without a real clinical trial, it's hard to say definitively, and we are all

about obviously, safety and efficacy. I just encourage anyone who reads or gets excited about any of these nutraceutical approaches that you have that conversation with your doctor before launching into taking anything.

Jason Menzo, Chief Executive Officer:

Thank you, Claire. Ophthalmologists or optometrists, we've got many great ODs across the country who are terrific at taking care of low vision and inherited retinal disease patients as well. So thank you very much. Thank you for the question. I also want to go back, something that our guest who asked the question live mentioned, which really warmed my heart, was getting involved with the chapter and getting back in touch with, in this case, Martha Steele, who is our fantastic chapter president in the Boston chapter. But anyone who's on the call today in the U.S., so this is one area that we have not yet expanded beyond the U.S. but we may be making some announcements in that regard shortly. But anyone in the U.S., in any market really in the U.S., we have chapters spread all over the country.

Our chapters are titled after one of our very special co-founders, Lulie Gund. Our chapters are named the Lulie's Next Chapter after Lulie Gund. And on our website, again at fightingblindness.org, you can do a quick search and find out where the chapters are within your local community, what the chapter is, any chapter events, any fundraising events, any educational events. There's so much happening really across the country. I was looking at it yesterday. We've got Vision Walks, chapter programs, days of vision seminars, special events, literally all over the country. Hundreds of them happening just this year alone. And so there's plenty opportunity for everyone on the call today to get engaged in one way or another. And for our friends from outside the US we've mentioned it a couple times. We have our international webinar. We put the link, how to register in the chat. But of course, like everything, you can find out more at our website fightingblindness.org.

As we wrap up today's call, I do want to remind you that if we didn't get to your question, and I know that we didn't get too many of them because there are so many that got chatted in today, we will be following up with every single one of you in the next couple of weeks. I do want to say a huge thank you to all of you

for spending time with us on this call. We really do aspire to be the global leader in driving the research for treatments and cures for inherited retinal diseases. And I think we're well on our way, and it's only possible for us to accomplish this and really for our whole community to accomplish this if we leverage the combined power of our entire community coming together to achieve these great things. The Victory for Vision is one example, but there's many others.

And so while as an organization we're having global reach, we really do think of it every single day, how do we put the interest of you, each individual person, front and center, and develop a relationship with you and be able to provide the resources that you need as an individual? It's important for us to continually evaluate how are we engaging our community and what structure best enables us to do that.

In the coming months, we're going to talk a little bit about how we're set up as an organization to both have massive global reach and drive the field forward, but at the same time have a personal connection with each of you in our community. So more on that to come.

We do really welcome your feedback and suggestions related to its webcasts or really anything that we're doing at the Foundation. And again, you can reach out to us directly at info@fightingblindness.org, and you can always learn more at our website fightingblindness.org. If you have any questions, don't hesitate to reach out. And I'm going to turn the call over to Chris to wrap things up for today.

Chris Adams, Vice President, Marketing & Communications:

Thanks, Jason. It's Chris Adams again. We'd like to thank everyone for joining and participating in today's call. As a reminder, there will be a transcript and audio recording of today's call in the next week available on our website at fightingblindness.org. And also as a way to stay connected, follow us on social, to stay informed on the latest news and activities from the Foundation. You can like and share the Foundation's posts on your own social media channels to help spread the word throughout the month. And again, if there's any other information you need, please reach out to us by sending an email to info@fightingblindness.org. Thank you and have a great day.