

**Foundation Fighting Blindness**  
***Insights Forum* Transcript**  
**August 12, 2021**

**Chris Adams, Vice President, Marketing & Communications:**

Good afternoon and welcome to the Foundation Fighting Blindness Quarterly Insights Forum. I am Chris Adams, the vice president of marketing and communications 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 it's available in closed captioning. To activate the closed captioning, please select the closed captioning option located at the bottom of the Zoom interface.

Please note that on today's call, all speakers do have their video live. However, all the comments will be provided verbally, and there are no slides. If you are using a screen reader, please be aware that the controls are at the bottom of the Zoom interface. This control bar may collapse when it'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 also ask questions through the Q&A and chat features, or by sending in an email to [info@fightingblindness.org](mailto: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 like to turn the call over to Jason Menzo.

**Jason Menzo, Chief Operating Officer:**

All right. Thank you so much, Chris and good afternoon, everyone. Thank you for joining us today. My name is Jason Menzo and I am the Chief Operating Officer here at the Foundation Fighting Blindness. It's my pleasure to welcome you all to our quarterly Insights Forum webcast. The purpose of this session is to update you on the latest developments here at the Foundation Fighting Blindness and

within our broader community. We have a very full agenda today, as we seem to every quarter. First, I'm going to highlight the significant progress we're making on engaging and educating our community, our fundraising efforts, our public awareness of our mission, and all things related to engaging those of you out in the marketplace and across the country.

I will then hand it over to our Executive Vice President of Corporate Development and Chief Business Officer, Peter Ginsberg, who will be providing a draft summary of our financial performance through June 30, 2021. He'll also highlight recent corporate partnership developments that have been undertaken here in the last few months. Our CEO, Dr. Ben Yerxa, will then share several updates related to two strategic initiatives, including our venture fund called the Retinal Degeneration Fund. And then following Ben, we are pleased to welcome to the call, Dr. Claire Gelfman, our new Chief Scientific Officer who joined the Foundation this past April.

I just want to take a minute to share a little bit about Claire. In her critical role, Dr. Gelfman is responsible for leading the overarching scientific strategy for the Foundation, including our preclinical and early translational research programs. She also serves as the primary scientific contact within the Foundation's Scientific Advisory Board, our Research Oversight Committee, also interacting extensively with academic institutions and relevant government agencies. On today's call, she'll be providing a summary of our recent developments in our science funding and outreach in that regard.

And then as Chris mentioned, after our formal remarks, we will have a Q&A period. And at that time, Chris will repeat the instructions on how to ask questions. And as he had mentioned, this call is being closed captioned and a replay and fully accessible transcript will be available on our website in the weeks ahead. As always, we welcome your feedback or suggestions related to this webcast or the Foundation in general. You can reach us anytime at the email address [info@fightingblindness.org](mailto:info@fightingblindness.org). And of course, you can always learn more at our website at [fightingblindness.org](http://fightingblindness.org).

I'd like to start today's call by highlighting a few key themes that have emerged out of the past year. They include flexibility, teamwork, and innovation. In the ongoing and evolving environment caused by the pandemic, we continue to have

to be creative and flexible. As you all know, we have converted nearly all of our fundraising efforts over the past year and a half to a virtual experience, including our recent National Virtual Vision Walk, which was held back in June. In that instance, we converted 20 spring walks that would typically take place live all over the country into one national event. I'm proud to report today that that national event raised over two and a half million dollars to help advance our mission. We continue to assess the situation related to in-person events. As of right now, we do plan on hosting 15 in-person outdoor Vision Walks this fall. Of course, for all information related to any of our events, you can find out more at our website, [fightingblindness.org](http://fightingblindness.org) in the Events section. That's where we will communicate if anything changes with regards to our plans.

Another key theme from this past year is teamwork. As we celebrate our 50th anniversary year, the message, "Together, we're winning," captures the essence of our many constituents who are all coming together and working as one, to together fight the diseases that cause blindness. It is not just about fighting these diseases, it's about celebrating the wins and our many victories along the way. As part of our year-long celebration, we launched the Victory for Vision Campaign, which is designed to be a strategic effort to deploy dynamic communications, high volunteer engagement, and general growth of our major gift and legacy programs. We set a bold fundraising goal for this campaign. Our objective is to raise an additional \$50 million over the next five years. We're starting with a key group of base donors. And the campaign is designed to increase the revenue that we're putting towards our mission above and beyond the annual fundraising efforts that we typically have. So that \$50 million we're planning to raise over the next five years is on top of the tens of millions of dollars we typically raise in a year already.

I'm really pleased to report that through the tremendous effort of our campaign volunteers and leadership and our internal staff, that already, we have gained commitment for over 40% of this ambitious goal, which is way ahead of schedule. And so thank you to everyone who's involved. And if you would like to learn more about the Victory for Vision, to find out how you can be a part of this really significant effort, please check out the website at [victoryforvision.org](http://victoryforvision.org). And that's the word for, F-O-R. On that website, you'll find new videos and information that details the campaign, and also interviews with leaders in our community

regarding the potential for treatments and cures driven by this additional funding. It's a really important initiative and I encourage everyone on the call today to engage and to learn more.

Another key initiative that requires teamwork and innovation is the launch of Lulie's Next Chapter for Light & Vision, which is our network of more than 40 volunteer-led chapters across the country. We are investing in new staff, new tools, new communication efforts to support our chapter leadership, and we are already seeing a dramatic increase in engagement across the country. By creating a process for volunteer connections, as well as localized outreach, we have been able to engage and grow a base of new constituents who are not currently associated with the chapter. This has led to over 100 potential new volunteer leaders across the country.

One of the ways that we've been able to grow the connection to our chapters is through our National Chapter Webinars. Our most recent webinar, which was just last month, had more than 1,500 attendees and focused on introducing the audience to the basics on clinical trials. The success and growth in participation for these webinars can be attributed to partnership. One of the themes that we're seeing right now is really teamwork and collaboration. And so it's an internal effort between our chapter team, our professional outreach team, and our market teams working together. Again, I encourage everyone who's participating in this call to learn more about Lulie's Next Chapter for Light & Vision by going to [fightingblindness.org](http://fightingblindness.org) and looking at the Chapter section. We have chapters all over the country and we would love to continue to invite all of you to participate within that chapter network.

The final theme I would like to highlight today is innovation. We are innovating in so many new ways, not just as it relates to the things that Dr. Gelfman's going to be speaking about in a few minutes as it relates to the science, but just how we're connecting with our constituents and bringing new ways to communicate and educate to life.

We launched a new series called Eye on Education, which is a series of webinars that is designed to educate new members to our community with introductory scientific information with a combination of live and on-demand programs.

Again, I would encourage everyone who has ever had a question about what is a clinical trial? Or I've heard the term rods and cones, but I don't really know what that means, or gene therapy versus cell therapy, what is the difference? What does this mean? To go to our website and to check out the Eye on Education seminars. They're about 20-minute webinars. They're really easy to digest the information. And I think that they really improve the way that even a lay audience can understand our science and what it is that we do to help advance our mission.

We also recently launched another communication tool designed to do a similar thing. It's called our Eye on the Cure podcast series, in which we provide scientific information, news, and insights from the world of vision and retinal disease. These podcasts are hosted by our own, Ben Shaberman, our Senior Director of Scientific Outreach. And they include conversations with guests from the research in vision communities. New podcasts roll out every other Friday. And they can be found on a variety of platforms, including anywhere that you would get your podcasts, including Apple Podcasts, Pandora, SoundCloud, Amazon, and more. To learn more about the podcast, you can again, go to our website, [fightingblindness.org/podcasts](http://fightingblindness.org/podcasts).

As you can tell, there are so many great things that are happening as a result of the collaborative efforts across the Foundation community. We are super passionate, but we're also impatient to continue to make progress across these broad initiatives. During these challenging times, our entire community has stepped up to keep the Foundation's mission moving forward in top of mind. And we thank everyone in the community for all their efforts to help us accomplish that.

With that, I would like to now turn the call over to Peter Ginsberg, who's our Executive Vice President of Corporate Development and Chief Business Officer. He's going to provide a financial and sponsorship update. Peter, the floor is yours.

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

Thank you, Jason. Appreciate those comments. So much activity here at the Foundation. An important part of our commitment to the retinal disease community is regular and transparent communication, including around our

financial goals and our performance. Today, I'll provide a brief summary of our financial position and then share also an update on our recent corporate sponsorships. A lot of activity there.

The Foundation operates on a fiscal year that runs from July to June. So we completed our fiscal year 2021 on June the 30th. Our audited financial statements will be available this fall and will be accessible from our website homepage in the about us section under Financial Reporting. I'm pleased to provide the following summary based on our preliminary financial results for the fiscal year 2021, ending June 30th. Keep in mind again that these results are not yet final since the year-end closing process remains in progress.

We are happy to point out that we expect to exceed both our \$21.2 million budgeted unrestricted revenue and our \$6.7 million budgeted net fundraising surplus for the year. So, ahead of our expectations both on revenue and net fundraising surplus. That's really important because the net result there is that we project the Foundation to be able to support \$7 million in new research through our performance this past year. We were able to fund all of our prior research commitments and also fund these new research commitments that Dr. Claire Gelfman will highlight later in the call.

For fiscal year 2022, we have budget for substantial increases to both revenue and net fundraising surplus with revenue expected to exceed \$27 million and net fundraising surplus expected to exceed \$9 million. So, substantial upticks expected in this current fiscal year on both revenue and net fundraising surplus.

A key driver of our growth last year and expected growth this year is a substantial expansion in our corporate sponsorships that play a key role in the Foundation's funding, alongside individuals and foundations. We actively connect with these leading and emerging companies in our field that help fund various Foundation initiatives. Along these lines, we're really excited that Janssen, which is the pharmaceutical companies of Johnson & Johnson, has become a Visionary Champion National Partner of the Foundation Fighting Blindness. Thanks very much to Janssen. And it's that support of key partners like Janssen that makes possible success of our programs, such as our grants that drive the research leading to treatments and cures for blinding retinal diseases, as well as our

genetic testing program that allows clinicians to order free comprehensive genetic tests and counseling for their IRD patients.

On that front, we've made remarkable progress in expanding the My Retina Tracker Registry and that related genetic testing program, increasing the number of Registry participants to more than 18,500 member profiles with genetic testing information provided by nearly 10,000 of those members. So, this Registry is a very important tool for the Foundation and our inherited retinal disease community. It's essential in helping us understand how common each type of retinal disease is, how each disease impacts people's lives, and how the disease progresses. Importantly, the Registry and our Foundation team help researchers and companies to efficiently find eligible patients who might be interested in participating in research studies and clinical trials. So, that's one of the keys with this Registry and genetic testing program is to connect patients with inherited retinal diseases with clinical trials developing therapeutics that might benefit those patients.

I'd like to conclude by thanking our outstanding staff members and volunteers for all they do in working toward our mission every day. This is the team that works closely with our community, connect our donors and sponsors with the researchers and companies finding cures and treatments for inherited retinal diseases. Now, I am pleased to turn the call over to our CEO, Dr. Ben Yerxa. Ben.

Dr. Ben Yerxa, Chief Executive Officer:

Thank you, Peter. And good afternoon and thank you for joining us on our quarterly update call. The Foundation serves a critical leadership role as a catalyst for driving increased research funding. We have a multi-pronged approach, which enables us to access as many sources as possible. I'm going to talk about innovative funding initiatives that are generating attention and commitment. First, there's our approach to philanthropic venture funding, which is our RD Fund, the Retinal Degeneration Fund, which invests in companies with projects that can be in clinical testing in 18 to 24 months.

The RD Fund was created to help accelerate life-changing outcomes for people with retinal degeneration through direct mission-related investments in

therapeutic companies. Today, investments have been made in Atsena, CheckedUp, Lookout, Nacuity, Nayan, ProQR, SparingVision, Stargazer, Vedere, and Vedere Bio 2.

We continue to see progress across our entire portfolio of RD Fund investment, but there are four companies in our portfolio that are in various stages of enrolling patients in clinical trial.

This includes Atsena, which is currently enrolling its Phase 1/2 trial in LCA1, Nacuity, which is currently enrolling in Phase 2 trial in retinitis pigmentosa related to Usher syndrome in Australia, and ProQR, which completed enrollment in its Phase 2a trial in Usher 2A. And finally, Stargazer, which completed enrollment in its Phase 2a trial in Stargardt disease.

In addition, you may have heard about Vedere Bio 2, which recently completed a \$77 million Series A financing. Vedere, based in Cambridge, Mass is a privately held emerging biopharmaceutical company that's leveraging mutation agnostic technology and novel AAV therapy approaches to restore vision in all patients with vision loss due to photoreceptor cell death. The company was founded by the leadership and research team behind the original Vedere Bio, which was acquired by Novartis in September 2020. The recent financing was led by Octagon Capital, who was joined by new investors, Samsara BioCapital and Casdin Capital, and Vedere's founding investors Atlas Venture, Mission BioCapital, and our own RD Fund. So, we're looking forward to further updates from Vedere as they leverage the next generation optogenetic approach to treating underserved retinal diseases.

Dr. Ben Yerxa, Chief Executive Officer:

The RD Fund continues to experience really high deal flow with many exciting opportunities that just need more capital. And you can find out more information online at [rdfund.org](http://rdfund.org).

While the potential for the RD Fund is significant, we realized that there's also a need for alternatives to venture capital. So we've collaborated to develop new federal legislation called the LOANS for Biomedical Research Act or H.R. 3437. This bill, with its growing bipartisan support, would authorize up to \$30 billion in loans, which we call BioBonds. It's like the well-established \$270 billion Green Bond



market. This would lead to the creation of a new innovative funding vehicle that relies on private sector investment, pension funds, and insurance companies rather than the government funding like the NIH.

Last month, there was an excellent New York Times feature story which describes the legislation and highlights our Foundation board director and BioBonds visionary, Karen Petrou, and her late husband and co-visionary, Basil Petrou. A link to this article is available on our website under the Foundation in the News section.

You can help this effort by contacting your congressional representatives and asking them to support this important legislation. More about BioBonds, including representatives' contact information, and even a letter template, is available at the website [BioBonds.org](http://BioBonds.org).

As I wrap up, I am pleased to introduce Dr. Claire Gelfman, our recent addition to the team as Chief Scientific Officer. Claire brings over 20 years of successful ophthalmic R&D experience, along with a proven ability to manage cross-functional teams of scientists and business professionals. Most recently, she served as vice president of pharmaceutical development at Adverum Biotechnologies. Claire completed her undergraduate degree in biology from Washington University in St. Louis, and a Ph.D. in biochemistry at the University of Texas at Austin, and a postdoctoral fellowship at the University of California at Davis. Now, I'd like to turn the call over to Claire to provide an update on our science and research programs. Claire, it's all yours.

**Dr. Claire Gelfman, Chief Scientific Officer**

Thank you so much, Ben. It's really great to be here. Having been in the ophthalmology field my entire career, it's truly an honor to have joined the Foundation, an amazing organization known as the world's leading private funder of retinal disease research. We do have a tall order in front of us to fund the best studies that will have the most impact towards a cure to treat inherited retinal diseases, as well as dry age-related macular degeneration. To accomplish this, we are funding across multiple fronts, from early preclinical research to translational studies, testing potential therapeutics and preclinical models to human study. All

of which are working toward the common goal to find the causes, the preventions, the treatments, and the cures across a broad range of retinal diseases.

It's indeed a great time to be in ophthalmology with all the amazing innovation and progress, but none of this could happen without the insight and expertise of our Scientific Advisory Board made up of the world's leading retinal experts who provide insight to the Foundation on research and clinical advancements, and importantly, to review our research grant applications. These key opinion leaders in our field volunteer their time to make a difference in the inherited retinal disease community, and we are incredibly grateful for their service and their commitment. They recently met and agreed upon our newest cohort of grant awardees, which will be announced in the coming weeks, including the individual Research and Clinical Awards, the Career Development Awards, as well as the Program Project Award.

We recently had our kickoff meeting honoring our fiscal year 2021 awardees of our Translational Research Acceleration Program, also known as TRAP. The goal of this program is to accelerate the movement of preclinical research towards an Investigational New Drug, or an IND, filing into human clinical trials to provide a robust and diverse pipeline of potential therapies to fight inherited retinal degeneration and dry age-related macular degeneration. At our kickoff meeting, the awardees shared a snapshot of what they will be working on. This includes the use of antisense oligonucleotide, which is a type of gene therapy that corrects the disease-causing mutation, which is being developed for individuals diagnosed with Stargardt. Gene therapy is also being used to deliver a uniquely designed inhibitor of complement activation, a process known to be present in dry age-related macular degeneration delivered by subretinal injection in an animal model that represents features of the human condition. Speaking of animal models, those with inherited retinal diseases are lacking, especially in non-human primates. And one of our TRAP awardees will explore the stimulation of neural regeneration in a non-human primate retina to potentially restore vision in late-stage retinal degeneration.

We're also happy to support the completion of pre-IND toxicity studies to advance a novel small molecule therapy for Usher syndrome type 3 into Phase 1

clinical trial. An alternative to gene therapy is gene editing or CRISPR, which is a subject of another grant award for autosomal dominant retinitis pigmentosa to promote the survival of retinal cells. Neuroprotection or protection of the retina from photoreceptor degeneration, leading to vision loss is being evaluated by repurposing an already available medication to slow the rate of retinal degeneration and preserve vision in a significant number of patients with IRDs.

The subject of another TRAP award will be focusing on optogenetics, where a light stimulated protein is delivered to the retina. Note that this therapy, like some of the others I mentioned, work independent of the gene mutation causing the retinal disease.

Speaking of optogenetics, I would like to highlight the recent Phase 1/2 PIONEER clinical trial that recently reported encouraging results. In May of this year, GenSight Biologics published initial results in the journal Nature, describing partial vision restoration for single patients in their optogenetics gene therapy trial. And the Foundation Fighting Blindness funded preclinical research that led to the initiation of this important clinical trial. We are still awaiting the results from the rest of the patients from this trial, but if successful, this approach has the potential to restore vision for people who are blind, not only from advanced retinitis pigmentosa, but also from other retinal conditions, including Usher syndrome, Stargardt disease, and dry age-related macular degeneration.

When Luxturna was approved in 2018 as the first gene therapy-based treatment for RPE65 associated Leber Congenital Amaurosis, it really opened up the field and established confidence that gene therapy could revolutionize the way that we treat genetic diseases.

In addition to the continued quest to identify genes that are mutated resulting in compromised vision, understanding the general pathological mechanisms that go awry resulting in retinal disease introduces yet another treatment paradigm, which increases our chance on goal, the goal of developing treatments that will either stop the progression or cure altogether inherited retinal disease. As we continue to execute on the science goals on our five-year strategic plan, we are relentlessly pursuing all possible avenues, including funding, education, and communication with our investigators, colleagues, and constituents. We look forward to providing further scientific updates to the early academic research

that we fund, the translational studies we award, and the clinical trials we support, including those through our venture philanthropic RD Fund. Jason, back to you.

**Jason Menzo, Chief Operating Officer:**

Awesome. Thank you so much, Claire, for joining us today. It is so terrific to have you onboard and leading some of the most significant initiatives here at the Foundation. I sincerely can't wait to work with you in the years ahead and continuing to progress and make tremendous impact related to our science and research programs.

So, it is right now just about 1:30 here on the East Coast. As you all know, this is a live presentation. And so we have about 30 minutes available for us to do question and answer. One of the things that I saw a little bit earlier in the presentation chatted in and I want to just make sure we reiterate to everyone here today, is that there are no slides for today's presentation. Myself and the other speakers, we do have our cameras on, but there are no slides. And so this is just an audio description of the narrative that we're talking about today. I want to turn it back over to Chris, to provide the instructions for how everyone who is here participating today can ask their questions.

**Chris Adams, Vice President, Marketing & Communications:**

Thanks, Jason. There are several methods for asking questions. First, you may access the Q&A and chat features located at the bottom of the Zoom control bar and type in your question. Second, you can ask questions verbally. To do so, please select the hand raising function on the menu bar at the bottom of the Zoom interface, and we will provide you with instruction to unmute yourself. You can also unmute yourself on your phone by hitting \*6. And third, if you're joined by phone for today's call, you can press \*9 to raise your hand. Pressing \*6 will mute and unmute your line. You may also submit your questions via email at [info@fightingblindness.org](mailto:info@fightingblindness.org). Again, that's [info@fightingblindness.org](mailto:info@fightingblindness.org). Please note that if there are questions that we aren't able to answer on today's call due to time constraints, we will follow up with you directly via email over the next week or two. Jason.

**Jason Menzo, Chief Operating Officer:**

Thanks so much, Chris. While we are compiling questions, although there's already been a number of questions that have come in. But while we're compiling and getting organized on the Q&A session, I just want to let everyone here know that in addition to our speakers from today's call, Ben, Peter, Claire, Chris, and myself, we're also pleased to have Dr. Amy Laster join the call, who's our Vice President of Science and Awards Program. She'll be joining us to answer some of the questions, and actually one of the first questions I'm going to pose to Amy in just a minute here.

I'd also like to take a moment to highlight some of the resources that are found on our website. And some of the questions that have actually come in already, I'm going to direct folks to specific areas of the website. But in addition to our website, [fightingblindness.org](http://fightingblindness.org), we have our Facebook page where we post a ton of great resources, information, we even have events on our Facebook page, including our successful Music to Our Eyes series. And we've got some updates to that coming in the weeks ahead. But we also have a very active Twitter handle and LinkedIn and Instagram accounts. These are all great resources for learning more about the latest developments in retinal disease and all the activities that are happening here at the Foundation Fighting Blindness.

And if you have a specific question about your particular scenario or diagnosis or particular disease of interest, or have genetic information that you need a little bit of help in terms of getting answers or finding a retinal doctor or a professional that can help you, you can find out more information on how we can help guide you to the right resources on our website, under the Stories and Resources section or the Retinal Education sections. And of course, we are always here personally for you to answer any specific questions, 24/7 at [info@fightingblindness.org](mailto:info@fightingblindness.org).

Let's start with some of the questions that have come in already. I'm actually going to answer the first couple myself. There are always, and today is no exception, a lot of questions about particular genes, particular diseases of interest, and specifically about where to find the latest in clinical trials. Our website has a lot of great information on it, but I want to point out specifically at the website homepage under the research section, we have a fantastic resource

called the Clinical Trial Pipeline. On the Clinical Trial Pipeline page, we list every single clinical trial that is currently approved by the U.S. FDA for clinical trial. It's organized not only by the particular gene or the area of disease research, but also who the sponsor is and the stage of research. So, it's a great resource. I would encourage anyone who has specific questions about clinical trials that are happening today to look at that section of the website.

And then the other question which came in and specific to an area of the website is, I referenced earlier on the call, that we have a great Eye on Education series, where we have these 20-minute webinars that are available on demand, 24/7, where you can spend 20 minutes watching a video that'll describe the basics of the anatomy of the eye, or what is a photoreceptor, what's the difference between a rod or a cone, or what is gene therapy, just the basics. So that way, a lay audience who's just introduced to this community has some resources to be able to get up to speed on the basics and science quickly.

Again, at fightingblindness.org under the Retinal Education section, there is a tab called Eye on Education video series. For those who use screen readers or who have difficulty navigating the accessibility of some websites, our website is fully accessible for screen readers and really easy to navigate. You can flip on contrast and make the font larger all with just the click of a button.

Hopefully, those resources are helpful. The next question I want to pose to Dr. Laster. Amy, there are a number of questions specific to Stargardt that have come in. And I wondered if you could potentially give a quick overview of what's happening in the clinical space specific to Stargardt and different strategies that are being explored to advance treatments for Stargardt.

**Dr. Amy Laster, Vice President, Science and Awards Programs**

Thank you, Jason. Yeah, so I want to talk a little bit about some of the preclinical studies that are happening with regards to Stargardt, as well as some of the clinical studies that are ongoing. The Foundation certainly has always invested in research to support Stargardt disease. Currently, we are supporting researchers to continue natural history studies to understand exactly what's happening in patients, how the disease progresses, and to look at clinical endpoints, as well as

potential biomarkers. We are also studying and funding researchers to look at the genetics behind Stargardt disease. Usually, Stargardt is a result of mutations in the ABCA 4 mutation. And so within that particular gene, there are a variety of mutations. We have researchers that are trying to understand what are those variants within the gene and how those can be addressed. We are also funding researchers to develop animal models to better understand exactly how treatments can be measure within individuals with Stargardt disease. Along with that, there's a number of studies also that are developing not just gene editing strategies, but also small molecule strategies in order to mitigate some of these effects of Stargardt disease.

Specifically in the clinic, there are several companies that are conducting clinical trials for Stargardt disease. As Jason just mentioned, on our website, we list all of those. I'll just mention a few of them. Acucela is doing a small molecule drug for Stargardt disease. As well as Alkeus, that's another company that's looking at a particular drug that will reduce the toxins that build up in the retina and break down the retina causing disease. There's also research that's looking at using even stem cells, creating a patch in order to restore vision for individuals with Stargardt disease. One of our portfolio companies, Stargazer, is also looking at small molecules to treat some of the pathways.

So there certainly is a lot of activity happening around Stargardt in trying to mitigate the effects of that. And the Foundation has been instrumental not only in some of the trials that are ongoing, but also in some of the preclinical work that hasn't made it to the trial yet, but that we have optimism that it will.

**Jason Menzo, Chief Operating Officer:**

Fantastic. Thank you so much, Amy. And as is always the case, there are maybe even more than is typically the case, there are dozens and dozens and dozens of questions that have been chatted in. So, thank you to everyone participating today for chatting these questions. We will get to as many as we can. However, and I know Chris mentioned it, but I want to reiterate it, that any question that is asked today that we don't answer live, we will take it back into the office, construct the answers and reach back out to you individually in the next couple of weeks. Don't be discouraged if we don't answer your question here live on the

call. We will get to as many as we can, but we will follow up with every question in one way, shape or form.

The next question I want to pose it to Peter. It is about some of the financial terms that you referenced. In particular, you referenced the term net fundraising surplus. A few of our audience members asked what does that mean. And so maybe you can just describe a little bit about that term and how that equates to us putting money to our mission.

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

Glad to, and I am happy to answer that question. There may have been others that were curious about this as well. Net annual fundraising surplus equals fundraising revenue minus operating expenses. And so fundraising revenue can be from individuals, companies, sponsorships, event, activities. And then we net out from that fundraising revenue, the operating expenses to get to the net annual fundraising surplus. As I noted this year, fiscal '21, which ended on June 30th, we think that number will be in the \$7 million range. Those are dollars to fund new research commitments. And those dollars will be combined with dollars on hand to fund roughly \$20 million overall this year on research projects that we hope and believe will lead to preventions, treatments, and cures for people affected by retinal degenerative diseases. I think that should clarify what the net annual fundraising surplus includes.

**Jason Menzo, Chief Operating Officer:**

Excellent. Thank you so much, Peter. You know, we always get a lot of questions, and today's no exception, about the Registry. We talk a lot about My Retina Tracker and our genetic testing program. Claire, I thought maybe it would be a good opportunity for you to share a little bit broadly about the Registry, why it's important, how individuals can get into the Registry and get access to free genetic testing.

**Dr. Claire Gelfman, Chief Scientific Officer**



Thanks for the question, Jason. The Registry is an essential part of what we do and it's only going to be as powerful as the information that is input into it. Our Registry essentially collects information from you, from anyone who has been diagnosed with an inherited retinal disease. We offer, through our Registry program, free genetic testing. This is our open-access program. And all it requires is for you to have been diagnosed from a physician, with an IRD, with an inherited retinal disease. And that gives you free access to get not only genetic testing, but also through our partners, to get one-on-one with a genetic counselor, to help you understand the results when you get them. And then once you have that information, then get it deposited into our Registry.

Our Registry is really important. We've talked a lot today already about companies and others who are running clinical trials. Well, part of setting up a clinical trial is finding the right patient population to poise that trial for success. If, for example, a trial is enrolling for patients with Stargardt or retinitis pigmentosa due to a specific mutation, this is a very valuable resource to feed quickly and efficiently the enrollment for a clinical trial, to get to that answer even faster.

We also get the question a lot, many times we have been diagnosed with an inherited retinal disease, but the mutation is unknown or the results are not quite totally conclusive. That information is also still important to go into our Registry because as more and more of these gene agnostic approaches, in other words, potential therapies that are not necessarily dependent upon your mutation, but rather on your diagnosis, that information will then poise you to be chosen for clinical trial, even though perhaps the mutation information was not known at the time of the sequencing.

In order to actually get the testing, operationally speaking, once your doctor has given you that diagnosis, then you can actually go to our website and under genetic testing, there are two important documents. One is for you to fill out and the other is one for you to give to your physician who made the diagnosis, who can actually order the test. It's really quite simple in that regard. I really cannot overstate the importance of getting genetic testing. It's going to really seed not only enrollment in clinical trials, but it also helps to capture the clinical course of one disease so that we can best enroll for success when our partners come to us and ask for access to our Registry for the enrollment of a clinical trial.

**Jason Menzo, Chief Operating Officer:**

Excellent. Claire, that was fantastic. I know that folks probably got a lot out of that. Really in practical terms, when we use the term genetic testing, maybe in a real practical, to lay audience, what does that mean? There's a couple of folks who've chatted in, just we say, get a genetic testing, in real practical terms, what does that entail?

**Dr. Claire Gelfman, Chief Scientific Officer**

Sure, absolutely. A sample of either blood or skin can be used to actually do the testing. That's the source of material that the testing is performed on, and it's a molecular biology test. There are a lot of DNA tests out there that can be done. We've heard a lot about, for example, polymerase chain reaction, PCR testing, in the COVID life that we live now. We can actually sequence, we can determine the sequence of DNA at different genes on different chromosomes from those tissue samples. And because we know the 250 genes that go awry in various retinal diseases, we can test for the presence of that specific gene sequence.

We talk a lot about base pairs, the nucleotides, and this is something you'll learn a lot more about when you access the webinars that Jason was just mentioning. But we can actually measure and detect the specific DNA sequence at a specific place on a chromosome, on a specific gene on a specific chromosome to see what exactly that sequence is that defines the genetic cause for the diagnosis that one has received from their physician. I hope that answers your question.

**Jason Menzo, Chief Operating Officer:**

Thank you so much, Claire. Again, I want to reiterate to everyone at home or participating in the call today. If at any time today or otherwise, when we're not having one of these Insights Forum web meetings, if you ever have a question about anything that we do, you can always reach out to us directly at [info@fightingblindness.org](mailto:info@fightingblindness.org). As I'm sure you've picked up on throughout the course of this call, there are so many resources that we have on our website, including where you can find a retinal doctor near you, how to get involved in

clinical trials, what clinical trials are happening right now in what areas. And then as Claire just mentioned, information about genetic testing. And so I just want to continue to remind everyone how much information is on our website as well.

Ben, I want to come to you next. There are, as always, a lot of questions about what's happening in industry. Of course, one of the most important initiatives that we have is the RD Fund. Specifically, there's a question about the Best disease program at Iveric, but broadly, I'm wondering if you could just maybe share a few words about what's happening with the RD Fund and some of the aspects of what's happening from an industry perspective.

**Dr. Ben Yerxa, Chief Executive Officer:**

Sure. Thanks, Jason. Hello again. I think from an industry perspective, we're seeing just a tremendous amount of activity in terms of companies investing in programs that are either close to or into the clinic. And so if you looked at the list of clinical trials going on, there's something like 42 or more, and that list is going to grow over time. We are trying to stay close to industry so we can track their progress. Iveric is a good example company, a company that has really become a powerhouse in our space. They do work in dry AMD, but they also have multiple IRD programs, including one for Best disease that they licensed from University of Pennsylvania based on work that we funded as a Foundation. I haven't listened to their latest conference call, but as a public company, they do give quarterly updates on their updated timelines. I think the Best program is slated to be in the clinic either at the end of this year or the early part of next year. But that information can be easily found on their website.

In terms of the RD Fund, basically the Foundation is positioning itself to be able to follow technology further down the road. We're not changing our direction at all, we're just going farther down so we can follow programs from academia into biotech and to not just provide funding, but provide assistance. Because the Foundation, with all of its resources, including the Registry, the clinical consortium, our Scientific Advisory Board, we're really in a great position to help and advise these companies that we invest in so they can be successful as possible. So, we continue to see a lot of interesting programs, majority of which are coming out of labs we've funded, which is very gratifying for a funding

program, but we do look at anything with scientific merit and consider these for investment. And we typically make about two investments a year. We're pretty selective, but once we get behind the company, we really stand behind them and we're prepared to invest with them all the way along the way. Hopefully, that gives that a decent overview.

Jason Menzo, Chief Operating Officer:

Thank you so much, Ben. And of course, Ben mentioned it during his formal remarks, but I'll use this opportunity to just remind everyone that we have another great website called the RD Fund, the RD Fund's website, which is [rdfund.org](http://rdfund.org), where at that website, you can learn all about the portfolio companies that we're invested in. Certainly, any news. There's a lot of news that has been coming out and will be coming out in the weeks and months ahead out of the RD Fund. And so I encourage anyone interested in learning more about our investments in industry and in advancing promising programs into the clinic to check out the RD Fund's website.

I'm going to come back to Claire now. Claire has a tremendous amount of experience in various aspects of the retina, but specifically, we have a number of questions about age-related macular degeneration. The difference between dry AMD and wet AMD. What do we fund? What don't we fund? How does the AREDS vitamin regimen affects dry AMD progression? With your background, Claire, I thought it might be worth taking a few minutes and just giving a quick overview on some of those aspects.

Dr. Claire Gelfman, Chief Scientific Officer

Thanks, Jason, no problem. When we hear about the wet form of age-related macular degeneration, we're usually talking about the more blinding form. When I say blinding, I mean, acute blinding, loss of vision very quickly because the hallmark in a patient who's been diagnosed with a wet form is bleeding in the eye that can compromise vision very quickly. So, that's the bad news. The good news is that there are approved therapies to treat that bleeding in the eye because the mechanism of that is known. There's a growth factor that is important in that

pathological process, and there were several companies that have approved products to treat that, and it is working in a lot of patients with the wet form of macular degeneration.

Now, the dry form is much more prevalent. But interestingly, one can have the dry form and not even know it. One can go to their ophthalmologist for a routine exam, their eyes will be dilated and their physician will notice some yellow spots. This is called drusen, that are located on the back of the eye, signifying the early form of dry AMD.

So the wet versus dry is really about that bleeding per se, that I was talking about. While we don't have that same process in the dry form, the dry form will lead to blindness. So there are several stages of the dry form. The most advanced stage called geographic atrophy is just that. They're the areas of atrophy at the back of the eyes that are not functional. And if those areas are not functional, then the neighboring photoreceptors cannot function and our eyes cannot track light coming in and send messages to our brain to interpret what it is we're trying to see.

So the dry form, there's a lot of work going on right now. There are a lot of companies that are looking to find a treatment for the dry form of macular degeneration, because there is nothing out there. There are no approved products for early dry form, intermediate dry form, or the late-stage geographic atrophy. The goal is to prevent the dry form from reaching the geographic atrophy stage. The one thing that has been evaluated though, is a cocktail of vitamins, the AREDS that you were just talking about. And this is a cocktail of a variety of antioxidants that has been evaluated to see how patients with various stages of dry AMD progression has been measured following the delivery of the oral intake of this antioxidant cocktail. And while it is not a cure at all for dry AMD, it's thought that it delays the progression of the dry form.

I'm not a physician by training, so I want to keep it in my swim lane, as they say, but AREDS supplements are typically prescribed as a first-line defense when one gets diagnosed with dry form simply because there's nothing else out there and has the potential to delay the progression. There is a lot of work going on in the space though. Unlike the inherited diseases that we've talked about where a single gene is responsible for the disease, in the dry form and the wet form as

well, just age-related macular degeneration in general, is considered to be what we call polygenic, probably multiple genes across; there's a lot of diversity in the genetic makeup of patients with that diagnosis. A traditional gene therapy would not be amenable because there's not a single gene that's responsible for the pathology.

But we're learning more and more about what happens in the disease state for dry AMD. And with that information, companies are identifying and manufacturing therapeutics that treat those pieces of the pathology. So we're really hoping that with all the companies in the space right now, that we will have a treatment soon that will go beyond just the antioxidant cocktail.

Now, we at the Foundation focus on in terms of what we fund, inherited retinal diseases, as well as the dry form of age-related macular degeneration. These are areas that are starving for treatments. We have to pick and choose. We have tough decisions sometimes about what we fund. And there are treatments out there for the wet form of macular degeneration, thankfully. We want to help patients in areas where there's nothing else out there. And so that's really part of our decision in not funding the work in the wet AMD. However, we're all about fighting blindness no matter the cost, and a lot of these therapies that we're talking about may be able to have cross-functional uses. And so we certainly connect with many key opinion leaders and leaders in the field of wet AMD as well. They're very much a part of our network, so that we're really all still working together, no matter the cause of blinding eye disease.

**Jason Menzo, Chief Operating Officer:**

That's an excellent statement, Claire. For everyone who's participating today first of all, I just want to say thank you for the many of you who are participating. We only have just a few minutes left, but there's just a couple of final comments that I wanted to make.

We've spent a lot of this call talking about genetic testing or My Retina Tracker program, our free genetic testing program. I just want to reiterate that there's been a lot of chat questions about the cost of a genetic test. The fact is, as many of you know, that if you were to pay out of pocket, a genetic test can cost several

thousand dollars. And so it is a costly proposition for an individual to take on themselves, but I just want to reiterate that is why it is so important for us to be able to offer free genetic testing and free genetic counseling as part of our My Retina Tracker, free genetic testing program.

We recognize that there's a cost associated with genetic testing. That's part of our mission that we believe firmly. Hopefully, we can continue to fund it. And so we're working really hard from Peter and Chris's desk to work on finding additional funding mechanisms. But you can find out more about our free genetic testing, open-access genetic testing program at our website, [fightingblindness.org/open-access-genetic-testing-program](https://fightingblindness.org/open-access-genetic-testing-program), which is complicated. But if you just go to [fightingblindness.org](https://fightingblindness.org), under Genetic Testing, there's a ton of information. Including the genes that are tested, it's one of the most comprehensive panels available in the marketplace. We partner with a world-class lab called Blueprint Genetics. And a ton of information is available on the website. And of course, as we've said multiple times on this call, if you ever have a question about any of these topics, you can reach out to us directly at [info@fightingblindness.org](mailto:info@fightingblindness.org).

Okay. Unfortunately, we are out of time. We have many questions we did not get to, but we will follow up, as we always do, individually, via email. I just want to say thank you to all of you for participating in today's call. And of course, thank you to our panelists and our leadership team, Ben, Claire, Peter, Amy, Chris. There will be a transcript and an audio recording of today's call on our website. So, if you want to go back and listen to anything that we've covered today that you didn't catch in real-time, or you want to share it with friends or family, it will be on our website within the next couple of weeks. If there's any other information that we can provide or anything else that we can help you with, please reach out to us by sending us an email at [info@fightingblindness.org](mailto:info@fightingblindness.org). Thank you so much for your time today and have a great rest of the day and a great week.