

Foundation Fighting Blindness
***Insights Forum* Transcript**
May 31, 2023

Amanda Bement, Chapter Engagement Assistant:

Hello everyone. Let's get started. Thank you for joining today's Insights Forum. Before we get started, I'd like to briefly review a few details for the call. Currently, all participant lines are muted and without video. If you are using a screen reader, please be aware that the controls are at the bottom of the Zoom interface. The control bar may collapse when it is not in use. If you would 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're using the Window commands, you can press the alt key, and if you're using a Mac keyboard, press command and backslash at the same time. Today's conference is being recorded and is available with closed captioning. To activate the closed captioning, please select live transcript at the bottom of the Zoom interface and then select show subtitles. It might also pop up in the more section at the bottom right.

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 this call, you may ask questions through the Q&A and chat features or by sending an email to info@fightingblindness.org. We will address questions towards the end of the call during the Q&A session. I would now like to turn the call over to our Chief Executive Officer, Jason Menzo.

Jason Menzo, Chief Executive Officer:

Awesome. Thank you so much Amanda, and thank you to everyone for joining us on this call today. I am excited, actually thrilled because as we're starting the call, I'm seeing a bunch of people in the chat already saying "Hi, from Maine. Hi, from Puerto Rico. Hi, from New York. Hi, from all over California." This is a global call. It

is really exciting to have our global community together today and thank you for joining us.

Very excited to welcome you to our quarterly Insights Forum webcast. As you know, we use this Forum 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 field. It's also a great place for us to share the research and development progress that is happening in the broader community.

We have a very full agenda today. First of all, Chris Adams, who you heard at the very beginning of this call, who is our Vice President of Marketing, will provide an update on our current marketing initiatives and the many ways that we're reaching out and engaging with stakeholders across the community. Then Peter Ginsberg, who's our Chief Operating Officer, will highlight recent notable corporate announcements and sponsorships, along with a summary of our fiscal year 2023 financial performance through the end of March. Then Dr. Claire Gelfman, who's our Chief Scientific Officer, will provide a snapshot of recent research and development and clinical data related developments.

And then I'll take the call back and I'll provide a few updates on several of our key strategic initiatives and also highlight some of the recent news and announcements that came out of the Association for Research in Vision and Ophthalmology meeting, called ARVO, which happened just a few weeks ago in New Orleans. And finally, to conclude our formal remarks, we're thrilled to have one of the preeminent researchers in our field as a guest speaker today.

We are very pleased to welcome Dr. Shannon Boye, who's a professor and Associate Chief of the Division of Cellular and Molecular Therapy in the University of Florida's Department of Pediatrics. Dr. Boye will highlight the progress being made in the IRD research and development field and especially at a company called Atsena Therapeutics where she was a co-founder. Atsena Therapeutics is advancing treatments for several different inherited retinal diseases, and today she's going to highlight some progress on LCA 1, X-linked Retinoschisis and Usher syndrome type 1B.

And then as we always do following Dr. Boye's remarks, we will open the call for your questions. We're very pleased to have several other members of the Foundation's leadership team joining us for the Q&A session today, including Dr. Todd Durham, who's our Senior Vice President of Clinical and Outcomes Research, Dr. Amy Laster, who is our Senior Vice President of Science Strategy and Awards, and then Dr. Rusty Kelley, who's the Managing Director of our Venture fund, the RD Fund. And with that, I'd now like to turn the call over to our Vice President of Marketing and Communications, Mr. Chris Adams.

Chris Adams, Vice President, Marketing & Communications:

Thank you, Jason. It has been a very busy quarter for us as we have had many initiatives in progress focused on increasing awareness, outreach, and engagement within our community and beyond. On today's call, I will highlight several of these initiatives and upcoming events.

Earlier this month, we announced a partnership with Athletes for Hope, a national nonprofit that assists athletes of all levels with community service and advocacy work. This partnership helps to build awareness for people with blinding retinal diseases throughout the athletic community by showcasing sighted and visually impaired athletes from all walks of life who share their personal stories of what life has been like through the changing lens of their own eyes.

Starting tomorrow, June 1st, we are launching a month-long awareness campaign called Spotlight on Usher Syndrome, culminating in Deafblind Awareness Week from June 25th to July 1st. Usher Syndrome is a genetic disorder that affects both vision and hearing in nearly 25,000 individuals in the United States. A majority of the outreach for this campaign will take place on our social media channels. In addition, we are working with several partner organizations as part of this campaign. We would like to thank Save Sight Now, Usher Syndrome Coalition, Usher Syndrome Society, and Usher 1F Collaborative for their participation.

Moving on to our chapter network, we continue to expand our chapters across the country with plans to grow from 44 to 58 over the next year. Our first four new chapters are in Miami; Oklahoma City; Portland, Oregon; and Portland, Maine. The volunteer community leadership teams are already planning chapter

activities and events. We encourage you to get engaged with your local chapter by visiting www.fightingblindness.org/chapters. If you're interested in helping to launch a chapter in your area, please reach out to chapters@fightingblindness.org.

The chapter's Vision Seminars are live and in person once again. These free half-day seminars provide the latest information on blinding retinal diseases and age-related macular degeneration, also known as AMD. Featured speakers share research, scientific advancements, current and upcoming clinical trials, the latest on genetic testing, and more. We will be hosting four seminars in the next 12 months, so be on the lookout for one in a city near you.

In addition, we are hosting a National Chapter Vision Webinar on cell-based therapies on Saturday, June 17th at 12:00 PM Eastern. This free virtual event will include an introduction to cell-based therapies provided by Ben Shaberman, the Foundation's Vice President of Science and Communications, as well as presentations by featured speakers including Dr. Henry Klassen, Co-founder and Chairman of jCyte, Dr. Kapil Bharti, Director of the National Eye Institute's Intramural Research Program, and Dr. Carl Wallen, Assistant Professor of Ophthalmology and Director of the Richard C. Atkinson Laboratory for Regenerative Ophthalmology at the University of California, San Diego.

In terms of other events, I'm excited to report that we've hosted 30 VisionWalks across the country this fiscal year, which raised over \$3.4 million, attracted more than 9,500 attendees and 750 teams. We have additional walks throughout the month of June, so get ready. These fun, family-friendly events bring together walkers across the country as we take steps toward treatments and cures for inherited retinal diseases. To learn more about our VisionWalk Program, please visit www.visionwalk.org.

Earlier this month, we hosted the New York Night for Sight gala event at The Lighthouse at Chelsea Piers in New York City. The Foundation presented its visionary award to three deserving honorees, Dr. Pravin Dugel of Iveric Bio, Avi Kaner of Morton Williams Supermarkets, and Doug Zarkin, formerly of Pearle Vision. Chaired by Foundation board members, Jason Ferreira and Evan Mittman, more than 300 people attended the event raising nearly \$750,000.

The St. Louis Night for Sight event was also held in May at historic Grant's Farm. Chaired by Foundation board member, Jason Morris, and Trustee, Michael Lowenbaum, more than 250 people attended the event raising nearly \$300,000.

I'd like to conclude my remarks by highlighting our new initiative under the Foundation's Raising Our Sights Do-it-Yourself fundraising program. It's called Vision Warriors, which relates to taking the challenge of ending blinding diseases and pushing beyond our limits through active and challenging programs to support the Foundation's mission.

This initiative includes events such as marathons, cycling events, obstacle course races, long-distance hikes, and triathlons. We have also designed a program that captures simple events such as a pushup challenge. It is our goal to raise awareness of our mission in front of new audiences and to provide a community with an opportunity to get physically active in ways that best suit them. If you have an endurance event in your local area and you'd like the Foundation to get involved, we encourage you to email raisingoursights@fightingblindness.org or visit the Vision Warrior section of the website under Ways to Give. I am now pleased to turn the program over to Peter Ginsberg, our chief operating officer.

Peter Ginsberg, Chief Operating Officer:

Thanks, Chris. In addition to the essential funds we raise through VisionWalks and other special events that Chris discussed, we collaborate with corporate sponsors to gain funding for important Foundation initiatives. We continue to engage with both leading and emerging companies in our field and beyond. And along these lines, we're really pleased to highlight our new Gold Outreach partnership with Regeneron, which is one of the world's largest biotech companies. Regeneron has seven FDA-approved medicines including EYLEA, a global leading treatment for wet AMD, diabetic retinopathy, diabetic macular edema, macular edema following retinal vein occlusion, and retinopathy of prematurity.

Regeneron has a June FDA review date for aflibercept 8 mg, which could allow for extending dosing intervals for this important medicine. We're very grateful for Regeneron's partnership along with all of our corporate partners that provide critical support to the Foundation and our research and outreach programs.

That brings me to our financial summary. The Foundation operates on a fiscal year that runs from July to June and I'll report on our financials through March.

For the first nine months of our fiscal 2023, our unrestricted fundraising revenue was \$16.1 million against operating expenses of \$12.7 million for a net fundraising surplus of \$3.4 million. We're tracking our overall budget plan for fiscal 2023 in which we're targeting a \$13.0 million net fundraising surplus to support new research funding. As we come to the end of this fiscal year, we're well into our 2024 budget planning process. Our focus remains on maximizing the funds we use to support important research by executing successful fundraising initiatives and by prudently managing organizational expenses.

Over the past year, there have been a number of significant outcomes from the many years of R&D investments the Foundation has made. In particular, I'd like to highlight some updates related to the Retinal Degeneration Fund, or RD Fund, which is the venture arm of Foundation Fighting Blindness. The RD Fund is focused on mission-related investments in companies with projects nearing or in clinical testing.

First, Atsena Therapeutics received FDA clearance for its investigational new drug, or IND, application for a Phase 1/2 clinical trial of its gene therapy focused on patients with X-linked Retinoschisis, or XLR5.

Also, Atsena recently presented positive six-month Phase 1/2 safety and efficacy results in Leber Congenital Amaurosis 1, or LCA 1, patients for a different gene therapy, and Dr. Boye will talk more about some of this encouraging data later.

Second, Opus Genetics received FDA clearance of its IND application to begin Phase 1/2 testing in LCA5. We look forward to the dosing of the first patients in that trial.

Third, Perceive Biotherapeutics received FDA clearance of its IND application to begin Phase 1/2 testing of its complement inhibitor in patients with dry AMD.

Perceive is one of our newer investments and we're excited to have a portfolio company focused on this large dry AMD population.

Fourth, SparingVision received FDA clearance of its IND application to begin Phase 1/2 clinical testing of its lead gene-independent therapy for the treatment of retinitis pigmentosa, or RP. We're really excited about all the new companies bringing therapeutics into human clinical trials.

SparingVision also just announced that a second program, a mutation-independent cone reactivation therapy for late-stage RP, has entered IND enabling studies with a regulatory submission plan for early 2024.

So, several positive milestones achieved recently by our RD Fund companies, but we also had a recent disappointment in our portfolio. The Vedere Bio II, which was launched two years ago to develop gene therapies for the eye, recently announced plans to wind down its operations. The results of recent pre-clinical efficacy studies did not meet the pre-established bar for success and thus the team made the difficult decision to discontinue its efforts.

While this is disappointing, many more programs fail than succeed at this stage of development, so were bound to have this type of news alongside the advancements I noted earlier. Importantly, this does not reflect on the early success and sale of Vedere Bio I to Novartis in 2020, and we're optimistic that the Vedere Bio I programs will continue to advance in Novartis's hands.

As we continue to support our existing portfolio company's progress and evaluate new investment opportunities, we're also adding expertise to the RD Fund team, and I'm pleased to announce and note that the RD Fund recently announced the appointment of Dr. Mark Blumenkranz to the RD Fund board.

Dr. Blumenkranz is a professor emeritus in the Department of Ophthalmology at Stanford and also chairman of an early-stage healthcare incubator and the recipient of multiple distinguished awards. He served as the Chairman of the Department of Ophthalmology at Stanford from 1997 through 2015 and is a skilled entrepreneur and venture capitalist. We are really excited to have Dr. Blumenkranz on our RD Fund team.

Finally, I'd like to highlight one other recent business announcement in our field. Last month, Astellas Pharma, a global pharma company and Foundation Outreach partner, announced plans to acquire Iveric Bio, a biopharma company addressing debilitating retinal diseases, that is also an Outreach partner of the Foundation. So, both of these companies are important Foundation partners and corporate sponsors. Iveric has multiple pipeline programs including ACP, which is a complement C5 inhibitor, for the treatment of geographic atrophy secondary to AMD. Iveric submitted a new drug application to the FDA earlier this year and a response is expected in August.

With those updates I'm now pleased to hand the program over to Dr. Claire Gelfman, our Chief Scientific Officer, to provide a snapshot of some of the recent developments on the scientific front.

Dr. Claire Gelfman, Chief Scientific Officer:

Thank you so much, Peter. Thank you, everyone. It's a really exciting time of significant progress on the scientific front with many new clinical data presentations and groundbreaking drug approval. In fact, there are now approximately 43 programs in the clinic with another eight INDs that have been submitted this fiscal year.

In February of this year Apellis Pharmaceuticals, which is a global biopharmaceutical company, announced that the FDA approved SYFOVRE for people with geographic atrophy, also known as GA, secondary to age-related macular degeneration.

AMD is a leading cause of devastating central vision loss in people over the age of 55 in developed countries. This newly approved therapy is the first of its kind by the FDA for geographic atrophy. SYFOVRE is delivered through an injection into the vitreous, which is the soft gel in the middle of the eye, and it's delivered once every 25 to 60 days in an eye doctor's office. It's designed to slow the progression of the geographic atrophy by inhibiting C3, a protein in the complement system, which is a pathway that is overactivated in patients with dry AMD.

Apellis also expects an approval decision for SYFOVRE from the European Medicines Agency in early 2024, and they have also submitted an application for approval to Health Canada as well.

There has also been excellent progress by Atsena Therapeutics, a company developing gene therapies for inherited retinal diseases. As Peter mentioned, earlier this month, Atsena received authorization from the FDA to launch a Phase 1/2 clinical trial investigating gene therapy for XLRS and plans to initiate the trial in mid-2023.

In addition, Atsena recently reported clinically meaningful improvements in vision from its Phase 1/2 gene therapy clinical trial for Leber Congenital Amaurosis 1, also known as LCA 1, caused by GUCY2D mutations, which results in early and severe vision impairment or blindness, and currently lacks an approved treatment. Atsena expects to report 12-month data later this year. Atsena also has a dual vector gene therapy in preclinical development for Usher Syndrome type 1B, which is caused by myosin-7a mutations.

We'll hear more about these exciting programs and the innovative vector-spreading technology today from our guest speaker, Dr. Shannon Boye, who is one of Atsena's co-founders.

Nanoscope Therapeutics recently reported results from its Phase 2b clinical trial using optogenetic therapy for people with advanced retinitis pigmentosa. The treatment is designed for people who have lost most or all of their photoreceptors, which are the cells that make vision possible. The treatment is delivered by intravitreal injection and uses a human-engineered virus to deliver copies of a multi-characteristic opsin, or MCO, gene to the bipolar cells.

These are cells that don't normally sense light but often survive after photoreceptors are lost due to advanced retinal disease. No goggles or glasses are used with this optogenetic approach.

Bionic Sight recently announced interim results from its ongoing Phase 1/2 dose escalation clinical trial for its emerging optogenetic treatment. There were meaningful vision improvements for RP patients receiving the highest dose of its

one-time treatment. A device that's worn like a pair of glasses, captures a scene a person is looking at and then generates a vision-enabling code, which is then sent to the light-sensitive ganglion cells and then onto the brain.

And then finally, Kiora Pharmaceuticals recently provided an update on its Phase 1/2 trial for an optogenetic-like chemical, that bestows light sensitivity to ganglion cells without the need for glasses or goggles. We only have data on the lowest dose administered thus far, but patients with advanced RP so far have shown an improved ability to perceive light and contrast. One gentleman commented he could see his wife's cell phone. Kiora's drug is delivered once a month and would require reinjection, likely on a monthly basis. Clinical studies that led to this clinical trial were funded by our Foundation.

In addition, PYC Therapeutics, an RNA-based therapeutics company, recently announced the submission of its IND for individuals with mutations of the PRPF31 gene.

Given our limited time today, I've only covered a snapshot of the recent promising clinical trial results, but I do encourage you to check out our website for frequent updates and a list of ongoing clinical trials at www.fightingblindness.org.

In addition, we have a great podcast series called Eye on the Cure hosted by Ben Shaberman, which provides scientific information, news, and insights from the world of vision and retinal diseases. With that summary, I'm now pleased to turn the call over to our CEO, Jason Menzo.

Jason Menzo, Chief Executive Officer:

Thank you so much Claire, and thank you to everyone for the participation. Again, as I said a few minutes ago, it's amazing to hear and see all the chats coming in from all over the world. Already a ton of questions have been chatted in or sent in via the Q&A. We will get to all those questions in just a few minutes.

As you can gather from all these terrific updates from Chris and Peter and Claire, this is a really busy time of year, both in the research and development aspect of what we do, but also as it relates to fundraising and engagement.

What I'm going to do now is step back and talk a little bit about some of the latest developments from the perspective of a few of our key strategic initiatives here at the Foundation.

I'm going to start with one of our more important collaborations that we have within the research and scientific community that occurs every year, and that is what we call the Innovation Summit for Retinal Cell and Gene Therapy. It's an event that we host every year in conjunction with the annual meeting of ARVO.

As I mentioned at the beginning of this call, ARVO took place last month in New Orleans. The many advancements in the inherited retinal disease field that we're hearing about on calls like the call we're having here right now, those advancements were on full display throughout the entire week at ARVO and the Innovation Summit, which was co-hosted by the Foundation Fighting Blindness and the Casey Eye Institute at Oregon Health and Science University. This brings together representatives from the biopharmaceutical industry, physicians, researchers, and many other stakeholders that are really key to this field.

The Summit is a great place for discussions of rapidly emerging therapies and strategies on how to move the most promising advancements forward into the clinic and through the clinic and ultimately, eventually into the market. This was our eighth annual summit, and again, the co-hosts were not only the Foundation Fighting Blindness, but also key people from the Casey Eye Institute, including Dr. Paul Yang and Renee Ryals, and our own Senior Vice President of Science Strategy and Awards, who we'll hear from in the Q&A session today, Dr. Amy Laster.

We had over 300 global experts in attendance and there were more than 30 presentations covering topics ranging from clinical trial design, optogenetics, gene and cell-based therapies. It was actually the first time that some data that had previously been conducted was presented publicly. It was a really special and inspirational meeting, and it felt great to hear the breadth of innovation and different approaches to retinal disease research that are happening in our field combined with outstanding scientific rigor. It's a reminder of how important the field and the work that we do is.

The Foundation will be issuing a summary of these presentations from the Innovation Summit and that will be made available on our website, which again is fightingblindness.org, in the weeks ahead. So, please stay tuned for information on this industry-leading research.

At ARVO, not only did we have the Innovation Summit that I just spoke of, but there are also several notable awards that were given out at ARVO in general. ARVO, which has about 9,000 - 10,000 attendees, gives out ARVO-wide awards, and it was really impressive that several people from our field in inherited retinal disease were recognized for some of the most prestigious awards at ARVO this year.

First, Dr. Eric Pierce, who many of you know, he's a world-renowned clinician-scientist. He's dedicated to treating inherited retinal diseases. He was honored with the very prestigious Proctor Medal, which honors outstanding research in ophthalmology, not just retina, not just inherited retinal disease, but all of ophthalmology. Dr. Pierce was recognized for his groundbreaking research efforts, many of which have been funded by the Foundation Fighting Blindness. They have focused on understanding the genetic causes of inherited retinal diseases and developing therapies to save and restore vision for people affected by them, including most notably the study at the Children's Hospital in Philadelphia, also known as CHOP, that led to the FDA approval of the RPE65 gene therapy, LUXTURNA.

Next, Dr. Budd Tucker, who's a highly innovative retinal disease therapy developer and manufacturing expert from the University of Iowa. He received the prestigious Cogan Award. And what's really cool about the Cogan Award is that it's a recognition that honors a researcher who's 45 years or younger and who has made important and worthwhile contributions to research in ophthalmology or in visual science that directly relate to disorders of the human eye or the visual system, and who shows substantial promise for future contributions to the field.

Again, this is not an award specific to inherited retinal disease or to the retina. It's really an industry-wide or a field-wide award and terrific to have both Dr. Pierce and Dr. Tucker recognized on that platform.

I have one final award and activity that happened at ARVO that I want to highlight, which was really special. This was the Helen Keller Prize for Vision Research, which was awarded by the Helen Keller Foundation in partnership with our friends at the BrightFocus Foundation for contributions to vision science.

This prize is given for research excellence as demonstrated by a number of significant research contributions to vision science during the course of a career or a single research contribution of exceptional importance to the field. This year's prize was awarded to four researchers, all from the University of Pennsylvania, that worked in collaboration. These researchers were Dr. Gus Aguirre, Dr. Jean Bennett, Dr. Al Maguire, and Dr. Sam Jacobson, who sadly passed away earlier this year, as many of you know.

We are thrilled with this well-earned recognition of the contributions by these outstanding researchers and physicians in the pursuit of cures and treatments for inherited retinal diseases.

During the Innovation Summit, there were a number of presentations about the many natural history studies that the Foundation has helped to design and fund . The latest development involved the launch of our Uni-Rare study, which is the first of its kind. I know we've talked about that study on previous Insights Forums as well, 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, including retinitis pigmentosa, LCA, Usher syndrome, Stargardt in the broad range of other conditions affecting our space.

Since the more common IRD-causing genes have been the subject of other clinical trials this specific trial, called Uni-Rare, focuses on the less common or more rare forms of IRD genes. The Uni-Rare study will help clinical researchers gain a better understanding of the course of progression of retinal degeneration for vision loss for people with mutated genes that have not been well-characterized in the clinic up until now. We are very proud of this highly inclusive study, which will allow and benefit a large segment of our population that is so genetically diverse that it 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.

The study is now open for enrollment, and if you'd like to learn more about this study and how you may be able to participate, please go to clinicaltrials.gov and type in the search bar, Uni-Rare. And of course, if you need help or have any specific questions about Uni-Rare or really about anything else related to what we do at the Foundation, you can always email us at info@fightingblindness.org and we can help direct you.

The final strategic initiative that I'd like to focus on today is our multi-year Victory for Vision Campaign. I know we've talked about this for a while over the last couple of years, and as a reminder, in 2021, we set a bold fundraising goal for this campaign, and the objective was to raise an incremental \$50 million over the next five years on top of our regular annual fundraising activities. So, it's incremental, on top of our annual fundraising, an additional \$50 million. The early returns were so positive and the tremendous efforts of our campaign volunteers, our leadership team, our staff, and many of you on this webinar today, who contributed to the campaign, we surpassed that original \$50 million goal pretty quickly, and then we raised the bar to \$75 million.

At this point in time, we're actually in the home stretch now of the campaign. We expect to announce the final results of the campaign later this year. I just want to take a moment to thank and really to express my gratitude on behalf of the entire Foundation community to the campaign co-chairs, who are David Brint, Robert Heidenberg, and Marsha Link, and also to the many, many volunteers all over the country and really all over the globe who have helped to make this such a successful campaign.

It is so critical to have the ability to bring in additional resources and additional funds to continue to fuel this rapid pace of advancement that we're seeing in the preclinical translational clinical stages of research. All of the comments that Claire and Peter made earlier in this call and that we talk about all the time are directly related to the fuel of dollars that come into the Foundation to help fund this terrific work. Our goal is not just to fund research, our goal is to fund research that will translate into potential future treatments and cures, get these treatments into the clinic, and we can only do that through the initiatives of things like the Victory for Vision Campaign.

For all of you on the call today who have already donated to the campaign, thank you so very much. And if you haven't yet participated, now is the time to support this important initiative. You can learn more or perhaps even make a donation directly to victoryforvision.org.

So, the time has come. It's my absolute pleasure to introduce our key speaker for today who's really at the forefront of driving the rapid pace of academic and clinical research. I think many of you know her name, many of you have probably heard her speak before. She's a great advocate for the patient community and a terrific researcher.

Her name is Shannon Boye. Dr. Boye is a renowned retinal researcher making invaluable contributions in the development of gene therapies for the treatment of inherited retinal diseases. She has a Bachelor of Science in marine biology and a minor in chemistry from Fairleigh Dickinson University and a PhD in neuroscience from the University of Florida. Dr. Boye serves as the professor and associate chair of the Division of Cellular and Molecular Therapy in the University of Florida's Department of Pediatrics. And she has been recognized with several awards including the Foundation Fighting Blindness Board of Directors Award. She's a Gund-Harrington Scholar and received a Gund-Harrington Scholar Award for her excellence in gene therapy research.

As we're going to hear today, as part of her career journey, Dr. Boye also co-founded Atsena Therapeutics, which is a clinical-stage gene therapy company developing novel treatments for inherited forms of blindness. In particular, these programs include treatments and development for LCA 1, XLRS and Usher Syndrome type 1B. Shannon, a very warm welcome to you, and thank you so much for being with us today. I'm going to turn the call over to you.

Dr. Shannon Boye, Professor and Associate Chief, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida:

Thank you so much, Jason, for that very kind introduction. Hi everybody. It's great to see you, some familiar names in the chat, shout out to Steve and Lisa and Miriam and all of the other names that look really familiar, and of course to my

fellow Floridians, and thank you to Chris, Peter, and Claire for all of their really exciting updates earlier. But I especially want to thank the Foundation Fighting Blindness for the opportunity to speak today. It's always an honor to have the opportunity to speak to the patient community. It is genuinely my favorite audience, and the FFB has always been one of my biggest supporters. So, I love giving back whenever I have the chance.

For those of you I haven't told this story to before, I really did get my career in this field off the ground because of the FFB. I was a junior investigator, I was fighting pretty hard to get out from under the shadow of a very, very large and famous previous mentor. And it's hard when you're in that position to get grants. I had applied for many grants, and it wasn't until 2012 that I applied for an Individual Investigator Award from the Foundation Fighting Blindness. And they took a bet on me - they gave me my first grant. I remember where I was when I got it, how happy I was.

But with that money, I was able to generate the preliminary data that I needed to compete successfully for a larger grant from the National Institutes of Health. And I've now kept that grant going as well as other grants. I'm currently funded from both federal agencies, big pharma as well as foundations, but I really have the FFB to thank for getting my independent research career off the ground. I also have to say, they've become a second family. Some of you in the chat and the other panelists today have become good friends of mine. We're all down in the trenches together and we share a passion for curing blindness, and that makes for truly special relationships.

I'm very grateful for that. But not only has the FFB been instrumental in my academic career, they've been really helpful in helping me to get at Atsena Therapeutics off the ground. This is a company I co-founded three years ago. Where that all started was, I was becoming a frustrated scientist. LUXTURNA had recently been approved, but it seemed to me like everyone was trying to apply the same recipe to cure every single inherited retinal disease and it wasn't working. I was witnessing things that were frustrating, like seeing business decisions override science decisions or technologies being partnered to big pharma and everything just slowing down.

I got really frustrated admittedly, and I remember sending a very verbose email to the former CEO of the FFB, Ben Yerxa, and just expressing my frustrations. And he wrote back just with a quick email saying, "Let's talk." And so, that was the beginning of a conversation that led to the start of Atsena, and I'll be forever grateful to Ben as well as to Rusty Kelley who's on today's call for helping me, mentoring me through the beginning phases of that process. I'm not a businesswoman by any stretch of the imagination, I'm a scientist. Their mentorship was very valuable to me.

But the bottom line is that in order to get these therapies over the line to get them from bench to bedside, they do need to be moved out of academic labs like mine into a company environment where there's enough capital to get things done. The RD Fund was one of the very first investors in Atsena in the spring of 2020. Again, to the FFB and the RD Fund, I'm forever grateful for the support that you've provided.

It was spring of 2020 as everyone on the call knows that was a crazy time. Covid had just hit. Everything that I did was virtual. All of the pitches that I was doing to venture capitalists were over Zoom and over Teams, but it was still a really economically prosperous time. Everyone was interested in funding gene therapy research. In the spring of 2020, we were able to raise about \$8 million in a seed round, and then in the fall of that same year, we were able to raise \$55 million in a Series A. That really enabled us to execute on three of our top programs, as well as some additional undisclosed programs. Today I'm going to focus on LCA 1, XLRS, and Ush 1B, and just tell you a little bit about where we are with each program.

LCA 1 - I like to call it my baby because I've been with it for 20 years. I started working on this program when I was a graduate student long ago, but this is one of the most common forms of LCA and it impacts about double the amount of patients that have the very famous RPE65 form. So, this form of LCA is caused by mutations in GUCY2D. Again, I've been with this program for a long time and I'm really excited to report that we finished, we completely enrolled our Phase 1/2 clinical trial, as Claire was mentioning earlier, this included 15 patients that received three ascending doses of our gene therapy. For this gene therapy, we

used an AAV 5 vector containing a photoreceptor-specific promoter to drive expression of our therapeutic GUCY2D construct.

At the end of all of the adult cohorts, we included one cohort of pediatric patients, and excitingly, I'm happy to report that the one-year visit from our last treated patient just happened last week. Results have been amazing to see. Not only did we have an extremely clean safety profile, but we also saw efficacy.

I'm just going to talk very briefly about the type of efficacy we saw. One of the tests that we performed and is performed in many other ocular gene therapy trials is called Full Field Sensitivity testing, or FST. Essentially what this means is a patient sticks their head inside a dome, a flash of light is presented to the patient, and they click a button to indicate whether they saw that flash of light or not.

And with that test, we saw significant improvements in retinal sensitivity around 10,000-to-20,000-fold improvements. Massively significant improvements. We also saw some improvements, significant improvements in BCVA in some patients. That's the Best Corrected Visual Acuity. That was not across the board, but we did see significant improvements in some patients. Importantly, we also saw improvements in visually guided behavior using a test that was also used by Spark to get LUXTURNA approved.

You may have heard of the MLMT maze. It's essentially a maze that the patient walks through in different lighting conditions. What's really important about this test is that the FDA has approved it in order to get these gene therapies across the line and to all patients. To get these drugs approved, if someone exhibits significant improvements in an MLMT maze, the FDA will tell them, "Okay, this drug is approved." It's an approvable outcome measure, is the bottom line. We were very happy to see significant improvements on our MLMT scores as well.

Those are all cold, hard facts, and numbers, but I think what stood out to me the most were the anecdotes that we heard from the patients that we've treated. They've been by far the most heartwarming. We had one patient that was treated in September, and subsequently about six weeks later, it was Halloween, and she was able to read the ingredients on her kids' Halloween candy for the first time. We've debated within Atsena if that's a good thing or not. We also had another

patient that was able to navigate outside the home at night for the first time because she was able to see the hatches in the crosswalk. We had another patient take advantage of their ability to see lights by setting up a series of LED lights in their home so that they could better navigate through their home.

And then we had one patient that saw a star for the first time, which was especially heartwarming. And most recently, we had a patient's mom send in a video of her daughter seeing snowflakes for the first time. And that was the kind of video that absolutely brings you to tears. It was amazing.

I have to say, when I was a student, when I was a postdoc, a junior researcher, I used to think that curing blind mice was rewarding, but seeing those kinds of videos just knocks you off your feet, it's another level. Now that our last patient has had their one-year visit, what that means for us is that we can hold an End-of-Phase 2 meeting with the FDA.

What that means simply is that it's an opportunity for us to review our data from the Phase 1/2 trial with the FDA and propose a pivotal trial design that will be acceptable for ultimate approval - important parameters to align on our study design, outcome measures, et cetera. But essentially the hope for an end result is an agreed to roadmap and thresholds for success that are required to bring this product to market where it will be available for all patients. Very excited with our progress on LCA 1.

Jumping quickly, we're going to move to XLR5, or X-linked Retinoschisis. This is a relatively common X-linked IRD affecting boys, and it's caused by mutations in the RS1 gene. This gene makes a protein that's secreted, but interestingly, it needs to be made in and secreted from the photoreceptor cells in order to properly perform its function in the retina. The target in this gene therapy are the photoreceptor cells. Boys present very typically when they're in early school age with a difficulty seeing the chalkboard, even though we don't typically use chalkboards anymore, you get the point.

But these boys go for a general eye exam and a quick picture of the back of the eye called the fundus image reveals a very characteristic spoke wheel pattern. And that spoke wheel pattern is in the center of their retina, in the macula, and

it's indicative of what are called schisis cavities. Those are literally separations between the photoreceptors and the underlying retinal neurons. And because they're coming separated from each other, they can't talk to each other as effectively. And so, you also see significant declines in retinal function in these boys.

Fortunately, though, they do not lose retinal structure until their 40s and more commonly their 50s or their 60s. So, we have a very wide treatment window between diagnosis and when that structural loss occurs in which we can safely deliver a copy of RS1 to those patient cells. Remember I mentioned that there are schisis cavities in the central retina. Because of that, we want to avoid surgically disrupting the central retina, but because photoreceptors are the target in this indication, we still need a way to very efficiently transduce photoreceptors.

Now with commonly used benchmark AAV vectors, you could only deliver therapeutic transgene to the region of the retina that you physically detached with the subretinal injection. That would've been counter-indicated in XLR5. We'd never want to detach that schisis retinal region. So, to tackle this disease, we're going to leverage a novel capsid that we've developed at Atsena called AAV.SPR, that stands for spreading. And this novel capsid is really unique because you can place it in subretinal injection bleb within the retina, but it spreads well beyond the margins of that original subretinal injection bleb.

And what that means is that we can perform a peripheral subretinal injection of AAV.SPR, and by virtue of that spreading behavior of the capsid, we can get therapeutic RS1 in the central photoreceptors where it's needed to cure these patients. And we can do it safely without having to detach the central retina. We've already shown that this is possible in normal non-human primates. We've done a number of studies showing that we can peripherally inject SPR and get central RS1 expression safely without having to lift that region of the retina.

We've also done extensive studies in a validated mouse model of this disease, the RS1 knockout mouse, which just like humans exhibits schisis cavities and reduced ERG, and we've shown the ability to completely resolve those schisis cavities and significantly improve retinal function. We also completed a GLP safety study in

non-human primates. This is a highly regulated study that essentially ensures that your drug is safe in a clinically relevant species.

As Claire mentioned, and as Jason mentioned, super excited to announce that Atsena recently received clearance of our IND application that stands for Investigational New Drug Application.

That clearance is the FDA's way of saying, "We like what you've done, you're good to move into clinic." At this stage, we're really excited to proceed. We've already assembled a number of US sites and we anticipate dosing our first patient in just a few weeks. So, super excited about the XLR5 program.

Following behind XLR5 is our gene therapy for Usher syndrome 1B. Ush 1B is caused by mutations in the myosin-7a gene. This gene makes a protein that's expressed both within the inner ear as well as in the retina. As a result, these patients are born profoundly deaf. They have balance problems, and they begin to progressively lose vision within the first decade.

It's lost from the outside in, in a retinitis pigmentosa-like fashion. But these patients can retain central islands of structurally sound photoreceptors for decades, providing us a window of treatment in which we can get a functional copy of myosin-7a to those remaining cells. One of the major hurdles we face in this program though, is that the CDNA is too large to fit within a standard AAV vector. So, to tackle this program, we're leveraging multiple technologies in Atsena's toolkit. First and foremost, we're using dual AAV vectors.

Put simply what that means is that we take the large gene, we split it in half, we deliver the front half with one AAV vector, the back half with another AAV vector. Those two vectors co-infect the same cell. They then find each other via a region of complementary sequence they recombine to form the full-length gene and then go on to make full-length functional myosin-7a. We've worked now both within my lab and now under the Atsena umbrella for years, a decade now to ensure the safety and efficiency of these dual vectors.

And we've shown them to be extremely efficient. In recent non-human primate experiments, we've actually shown that we can drive higher than wild-type levels

of myosin-7a expression with our dual vectors packaged in AAV.SPR. What I mean by that is that we compared the amount of myosin-7a that's expressed in a normal monkey to the amount that's expressed in an AAV-treated monkey. And we're actually seeing higher levels in our AAV-treated monkey, which means we can bring down those doses, which is an added safety feature for patients.

More importantly, these dual vectors have been extremely well tolerated in non-human primates. That's also important for moving this forward. Secondly, we're leveraging that laterally spreading behavior of our AAV.SPR capsid because as I mentioned, most of these patients retain central islands of precious foveal photoreceptors that the last thing we'd want to do is surgically disrupt. We're going to be placing our dual vectors packaged in AAV.SPR in the peripheral retina, and by virtue of its spread, allowing for myosin-7a expression in the central photoreceptors where it's needed. This is a little bit behind the XLR5 program, but we continue to advance it into IND-enabling studies, which will start soon and pave the way to the submission of an IND.

Those are the top three programs. I think it's worth mentioning too, that outside of these three main programs, we continue to work on our platform technologies as well as on additional undisclosed preclinical programs. And I think the platform technology is really what differentiates Atsena from a lot of other companies that are out there because not only are we focused on specific programs and specific genes, but we continue to optimize and identify the best tools with which to tackle each indication, large or small.

And many of those technologies can be applied to many different genes. For instance, the AAV.SPR capsid that laterally spreads, I feel firmly that this is a capsid that could be deployed to any subretinally delivered gene therapy for a photo receptor-mediated disease. Our dual vectors, for instance, could be applied to other large gene diseases. That focus on technologies is I think what makes Atsena really stand out. But taken together, our growing team continues to develop novel tools with which to most effectively address these and other blinding conditions.

Before I wrap up, I just want to say again, thank you to everybody who attended today. I want to thank the FFB again sincerely for their commitment to my work

both academically as well as in Atsena for all the support you've given me over the years. And I especially want to thank the patients and their families who have continued to inspire me to do this work. I'm extremely happy to know you. I'm extremely happy, lucky to have the job that I do, I love it. Thank you so much for your time and I'm happy to answer any questions that you might have.

Jason Menzo, Chief Executive Officer:

Thank you so much, Shannon, and we love you too. It's so heartfelt and we really do appreciate the work that you do. And also, of course, participating on calls and forums like this. I do want to share with the broader team who's listening in today. There have already been way more questions asked than we could possibly answer in this setting. And that's okay. You have our commitment that we are going to follow up on every single question. I'm going to ask Amanda in a second here to reiterate how to ask questions.

We've got a lineup of a handful that we're going to cover here in just a minute. I do want to share, typically we end this call right at the top of the hour. We'll go a few minutes over today just to make sure we can cover a good handful of the questions that have already come in. But before we do, Amanda, can you please reread the instructions on how folks can ask their questions?

Amanda Bement, Chapter Engagement Assistant:

Thanks, Jason. There are several methods that you can use to ask questions. You can submit them through the Q&A or chat function at the bottom of your Zoom screen and make sure you include your name so that we can follow up afterward, as Jason said. You can also send an email to info@fightingblindness.org and we will follow up in the next week. Thanks, Jason.

Jason Menzo, Chief Executive Officer:

Based on how many questions we've got, it may take a couple of weeks this time around, but that's all good. Shannon, I'm going to ask the first question back to you - actually going a little deeper on the spreader or spreading vector. This question comes from Dan Day, one of our really key trustees down in Florida

who's been super involved with the Foundation. "Could you describe a little bit more about the expected clinical benefits of the spreading vector and a little bit deeper into what you expect the benefit to be?"

Dr. Shannon Boye, Professor and Associate Chief, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida:

Absolutely. First, hi Dan Day. I know him well. Good to see you. Good to hear from you. So, yes, the AAV.SPR capsid is really unique. Essentially the science behind it is that it doesn't bind to the extracellular matrix, so it allows the capsid to be more slippery and disperse itself more effectively in the subretinal space. But where this is going to become super clinically relevant is that it allows for treatment of the central retina without the need to actually detach the central retina. And while that is tolerated in some patients, there are many patients that have fragile retinal conditions where that's not well tolerated.

This is going to allow for safe treatment of central photoreceptors. The other thing that it allows for is for treatment of a wider area of the retina, which is always a benefit when it comes to improving your visual field. The more retina you can treat, the better that you'll be. I think those are the two main benefits. It's also very potent, so it allows for us to come down and dose and deliver less drug to a patient, which is always an added safety feature.

Jason Menzo, Chief Executive Officer:

Perfect. Thank you. I want to take a second just to invite my colleagues also. So, all of you, Todd, Claire, Amy, who else is here? Peter, Mark, Rusty, all of my colleagues who are here for the Q&A session, please come off camera and unmute your lines. I'll be directing questions to each of you in just a few minutes here. I'm going to start with you, Todd. So, we talk a lot about the patient registry, My Retina Tracker. We have several questions about, "Can you remind me what My Retina Tracker is? Why is it important to get genetically tested if I've been genetically tested in the past, but there was not a pathogenic gene variant found? When should I get retested?"

Maybe you could just kind of give a brief overview of the registry and some of those more common questions that we get about the registry.

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

I sure will. Hi everyone. This is Todd Durham. As Jason just alluded to, the foundation hosts My Retina Tracker registry. This is the largest retinal disease patient database that connects people to relevant clinical trials and natural history studies. We currently have over 24,000 participants in our registry, and over half of these individuals have genetic testing data associated with their profile. This is important because this information is critical to supporting the clinical trial process. In fact, a recent study we did, we found that more than a quarter of the current ongoing clinical trials leveraged our registry to assist in recruitment.

So, that's the compelling reason to participate in My Retina Tracker registry. And just briefly, I'll cover the issue of genetic testing. Our program offers no-cost genetic testing to individuals with diagnosed inherited retinal diseases. There are eligibility requirements. If you have already had your condition diagnosed, that is a gene identified as causing your condition, there's no need for you to be retested. And that applies to about 60 to 65% of people who have been tested with a very large clinical panel like the one we use in our program.

If you have been tested in the past with a smaller panel, something with maybe only 30-so genes, and that's happened more than three years ago, you would be eligible for testing under our current program because our panel has a larger set of genes, and we may be able to identify your causative gene if it hasn't been identified already. And there are numerous situations that are in the gray zone where it requires expertise to know when to be retested.

I would encourage you to speak with a genetic counselor who counseled you or your inherited retinal disease specialist who looks after you on a regular basis.

Jason Menzo, Chief Executive Officer:

Thanks, Todd. And I would only add one comment, which is again, any of these topics that you may have further questions about or if you're unsure if you had a genetic counselor that reached out to you after you had your test from another provider, don't ever hesitate to reach out to us directly and we can help advise. And again, you can contact us at info@fightingblindness.org and then we will make sure the right person on our team gets the information.

Thank you very much for that, Todd. I also want to make one other comment before going to the next question. A lot of the subject matter that we're talking about today, and I've seen it chatted in from a couple of different folks, can be perceived as pretty technical. And we recognize that this is a technical field and some of the information is technical just by nature. We try to make it as appropriate for a lay audience as possible, but sometimes the subject matter is just technical and that's just the way it is.

Of course, if we can help talk through it, don't hesitate to reach out again directly to us, or I'm going to point you to a great resource that we've got on the website at fightingblindness.org. Across the top, there's a section called Retinal Education, and under the Retinal Education tab on our website, there's a ton of information, both articles as well as videos that are really specifically designed to take this complex information and break it down into very easy to understand, especially if you're new to this field and you're newly diagnosed, you have a family member who's newly diagnosed, it can be a little overwhelming. We recognize that. We want to provide these resources to make it as easy as possible to educate yourself and become up to speed on what's happening in this field.

With that in mind, we're going to go to the next question. I'm going to direct it to you, Claire. Claire is our chief scientific officer. I have a question from Sherry Rogers in New York. Hi, Sherry. Great to have you on the call. Sherry asks about gene agnostic, she didn't use that term, but basically, therapies that are not specific to a particular gene.

So, perhaps therapies that may be beneficial to groups of people who have a variety of different genes. We hear about optogenetics as one approach, but

maybe you could talk about what are optogenetics and what are other gene-agnostic approaches to treatment.

Dr. Claire Gelfman, Chief Scientific Officer:

Thanks, Jason. This is Claire Gelfman again. So, there are a lot of companies, as I mentioned, that are using optogenetics as a way to treat inherited retinal disease. And the basis behind that is due to the fact that in late-stage photoreceptor disease, the rods and the cones in the back of the eye can no longer function. So, when we think about a gene therapy, they're just not there to receive the therapy. We're able to take advantage of the fact that even though during late-stage disease photoreceptors are dying, there are other areas of the retina, the inner retina, that are thriving and they can receive different types of therapies.

We actually give that light sensing capability that's normally found in photoreceptors to areas of the retina that are not degenerating, and that is the optogenetic method that is being used by Nanoscope and Ray Therapeutics, and GenSight, and several others that I mentioned earlier. And this is very important because it's a treatment option for those who have late-stage photoreceptor degeneration, and it's not necessarily dependent upon knowing one's causative mutation.

In addition to optogenetics, there are other, what we call gene agnostic approaches that are based on knowing about the method of degeneration of photoreceptors, and there are different companies that are treating the pathology of a dying photoreceptor as opposed to the genetic cause. And we're going to be hearing lots more about those in the coming years.

Jason Menzo, Chief Executive Officer:

Thank you so much, Claire. We're going to shift and talk about a couple of specific areas that we did get a bunch of questions on. So, there was this handful on EYS. Todd, I'm going to come back to you. I know we have some information we can share related to the EYS gene and then also maybe Amy from your perspective, anything from a research side of things related to EYS that you want to add or speak to. So, Todd, we'll go to you first and then to Amy.

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

Yeah. Hi everyone. Todd Durham here again, as many of you know, mutations or variants in the EYS gene are implicated in many cases of recessive retinitis pigmentosa. Therefore, it's a fairly common condition among individuals who are familiar with RP. I wanted to remind everyone that we are funding and sponsoring a natural history study that is an observational study in EYS-associated retinitis pigmentosa. The purpose of these studies is really to understand and describe the rate of progression, of vision loss and also structural aspects.

So, we look at the photoreceptor integrity over time, all with the goal of helping companies design clinical trials that are going to be successful. It helps us understand when we should intervene with various therapeutic approaches and understand how long and how large those clinical trials should be to demonstrate the effect that we desire to see. And then I think there are other aspects of EYS that maybe Amy can cover. I'll just pitch it over to her.

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

Thank you, Todd. This is Amy Laster, and we do currently have funding for an investigator group at Mass Eye and Ear that is looking at a therapeutic strategy for EYS-associated retinitis pigmentosa. Just as Dr. Boye mentioned, oftentimes you'll have some genes that are really too large to fit in the traditional AAV gene therapy. And so, what this group is particularly looking at is an alternative to gene therapy using what is called exon skipping. Basically what it is, is a strategy to skip over the mutation.

Instead of putting in a healthy copy of the gene, which is too large, just using tools, things that we've heard about like CRISPR or other editing tools that'll skip over that mutation so that the actual protein that is produced is a functional protein. We're excited about these kinds of therapies and as Todd mentioned, having the natural history study and data from that to speak to which subpopulations will benefit best from these therapeutic strategies are really complimentary to advancing these therapeutics.

Jason Menzo, Chief Executive Officer:

Thank you, Dr. Laster. A couple of questions and then we'll probably wrap up in the next five to 10 minutes. And again, I do want to reiterate that any question that we don't get to here, which will be many, we will follow up with you individually one-on-one in the next couple of weeks. And again, if you have a question that we didn't get to or if you haven't asked yet, you can always contact us at any time at info@fightingblindness.org. Rusty, I want to shift to you, Dr. Kelley.

Rusty is the managing director of the RD Fund. We've spoken about the RD Fund at length over the last several Insights Forums, why it exists, why it's important, how it is an additional vehicle to help drive our mission into the clinic through venture investing and companies doing this work. Rusty, maybe you can give just a brief overview of what's currently occurring within the RD Fund. A lot of folks are curious about that. And then one of the things that you've looked at extensively in the RD Fund also is the Stargardt field. And so, perhaps you can answer some questions. We've got a ton about what's happening with Stargardt, and you're really well positioned to speak to that.

Dr. Rusty Kelley, Managing Director, RD Fund:

Thank you, Jason. Rusty Kelley here. Let's start with the second question first. I noticed the many chats & questions with regard to that indication ABCA4. It's a really exciting time to be optimistic about all of the activity that's going on in Stargardt disease. We have a couple of colleagues within FFB that closely track all programs within Stargardt - preclinical and clinical development. And there's currently 23 to our knowledge, and they span all modalities, whether it be small molecule and a variety of small molecules that there's multiple targets including the visual cycle and other related targets.

But there's the cell-based therapies, there's the DNA and RNA editing strategies. There's antisense oligo, and there's optogenetic strategies, and there's a number of gene agnostic approaches that are reaching later phase trials. So, very exciting time. We have a couple of ABCA4 programs within our portfolio. I'd just say many of the details have been kept confidential, so I won't share any details today, but

please feel free to reach out to me anytime, rustykelley@rdfund.org, and I'm happy to discuss more detail with you.

In terms of new and exciting activities, the strategy for the RD Fund currently is to really make a larger splash in dry AMD, which as Jason mentioned earlier, we've made an investment, or I think it was Peter that made the statement that we've made our first dry AMD investment in Perceive Bio. And so, we're very excited about our ability to get into those opportunities with a terrific investor base, management team, and then, of course, having that ground floor visibility into our companies that are managed by the RD Fund board and its management team.

And I have to say, among all these exciting activities, including the next wave of genetic medicine platforms, which you'll soon learn more about as we expect to make a couple of new investments in that area, allow me to make the shameless plug that in addition to Peter Ginsberg and Jason Menzo and Claire Gelfman of the RD Fund management team, we're under terrific leadership with the RD Fund, starting with the RD Fund Board Chair, Adrienne Graves, and with an outstanding board of directors, very diverse, cognitively and with domain expertise. Kelly Lisbakken and Jonathan Steinberg, Jean Bennett, Cathy Bowes Rickman, Jacque Duncan, José-Alain Sahel, Warren Thaler, David Brint, Tony Adamis.

And as Peter indicated earlier, we've been able to add Mark Blumenkranz, yet another terrific luminary to the RD Fund board. And then with Jason Morris, an FFB board director observing, and of course the great Gordon Gund, as well, is also active. So, we're under great leadership. We've been through one of the worst biotech downturns in the history of the capital markets, and we're well positioned to not only support our existing companies but to make new investments as well.

Jason Menzo, Chief Executive Officer:

That's great. Thank you so much, Rusty, it's fantastic. We're about 10 minutes past the hour right now. We're just going to take a couple more minutes. I've got one other question I want to get to, and actually maybe Shannon, maybe I'm going to put you on the spot real quick before we get to that last question. With

having you here on this call, it's such a great treat to have your perspective. If you were to, not by name, not by company, or anything like that, but outside of the work you're doing with Atsena, what gets you most excited about what's happening in the field?

Dr. Shannon Boye, Professor and Associate Chief, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida:

I think the gene editing space is a really exciting space right now. I heard many comments from colleagues of mine that went to the American Society for Gene and Cell Therapy meeting this year that said what was new six months ago is already old, it's just moving at such an incredible pace. And you're at a stage now where you don't have to make double-stranded breaks in DNA, you can do it more safely by creating a single-stranded break, and there's much fewer off-target effects. But gene editing is just so neat because it's getting at the root cause of the problem. You're going in and you're using genetic scissors to basically fix the genetic mutation instead of coming at it from, "Okay, let's deal with the effects, let's deal with the buildup of bisretinoids, you're going to the root cause of the indication. I think that's what excites me the most.

Jason Menzo, Chief Executive Officer:

That's great. Thank you for that. One final question, and then we'll move to closing remarks. A lot of questions about a specific gene, the PRPH2 gene. Amy, I'm going to ask you to speak a little bit about our activities as a foundation with regard to PRPH2, both as it relates to engaging in the community, but also as it relates to programmatic investments and new awards in that field. So, I'll turn it over to you, Amy.

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

Thank you, Jason. Again, this is Amy Laster, I'm really excited to talk about this. Just a couple of months ago, the Foundation along with the University of California San Diego's Shiley Eye Institute and the Nixon Visions Foundation hosted a workshop where we really brought together over a hundred scientists,

patients, family members, and industry professionals, really with the goal of engaging and building the scientific and patient peer PH2 community, sharing knowledge, and to launch a PRPH2 funding initiative. It will be funded by the Nixon Visions Foundation but managed by the Foundation Fighting Blindness to really fill the gaps with regard to PRPH2-associated mutations, which can lead to retinitis pigmentosa, pattern dystrophy, and other types of retinal dystrophy.

So, there truly is a need here to understand more about the disease with the ultimate goal of developing targeted therapeutic strategies. And even to that last point ahead of launching calls for PRPH2 research and clinical studies, the foundation recently awarded a group at Oregon Health Science University to look at one of those editing strategies that Dr. Boye just talked about in order to address PRPH2-related dystrophy. So, we are really excited about where the field is going and being able to come back to you soon and talk about potential therapeutic strategies for this retinal dystrophy.

Jason Menzo, Chief Executive Officer:

Thank you, Amy, and again, thank you to everyone for your participation today. Before I turn it back over to Chris, I'm going to make two final comments. Number one, I am so heartened by the level of engagement amongst our community, not just here on this call today, but really just in everything. We go to a VisionWalk, there's more people showing up at VisionWalks than ever before. We have a webinar, there's thousands of people on a webinar. The amount of engagement from the community is really spectacular. And again, today is an example, we've got people on this call from all over the world.

We think of ourselves as a global organization. We really are the undisputed global leader in the space, driving the research to provide for treatments and cures for inherited retinal diseases and dry AMD. And so, it's really a terrific thing to have the global community connect with one another in a forum like this. Thank you for participating.

The second thing that came up in a lot of the questions, and I just want to point to a resource, is, "This all sounds great, so much information. I don't even know how to digest it all." I mentioned it a few minutes ago. There's a tremendous amount

of resources we have on fightingblindness.org at the website. We've got a whole section on retinal education where there are videos, articles, Claire mentioned the Eye on the Cure podcast, which is available anywhere you get your podcast, be it Apple, iHeartRadio, Spotify, any of them.

And the other resource I want to point to is what we call our clinical trial pipeline. Again, on our website, there is a header that is titled research. And under the research tab, there's a section there called Clinical Trial Pipeline. If you click there, it goes to all of the clinical trials that are in the market right now, and it's something we update constantly. It's a great resource where you can find out what's happening right now in real-time based on the different genes or the different types of strategies, specifically advancing treatments and cures for inherited retinal diseases and dry age-related macular degeneration.

Again, if you have any questions, don't hesitate to reach out, and I'm going to turn it over to Chris to wrap up the call.

Chris Adams, Vice President, Marketing & Communications:

Thanks, Jason. We'd like to thank everyone for participating in today's call. As a reminder, there will be a transcript and audio recording of today's call within the next week on our website, www.fightingblindness.org. And also, be sure to follow us on Facebook, Twitter, LinkedIn, Instagram, and TikTok to stay informed on the latest news and activities from the Foundation. You can like and share the foundation posts on your own social media channels to help spread the word throughout the month.

If there is 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.