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AGTC - Q4 2019 Applied Genetic Technologies Corp Earnings Call

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PRESENTATION

Operator

Good afternoon, and welcome to AGTC Fourth Quarter and Fiscal Year 2019 Financial Results Conference Call. Today's call is being recorded.

Before we get started, I'd like to remind everyone that during this call, AGTC may make forward-looking statements, including statements about the company's financial results, financial guidance, its future business strategies and operations and its product development and regulatory progress. Actual results could differ materially from those discussed in these forward-looking statements due to a number of important factors, including uncertainty inherent in the clinical development and regulatory process and other risks described in the Risk Factors section of AGTC's annual report on Form 10-K for the fiscal year ended June 30, 2019.

For introductions and opening remarks, I'd like to turn the call over to Sue Washer, Chief Executive Officer of AGTC. Please go ahead.

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Good afternoon, and thank you all for joining us today. With me on today's call is Bill Sullivan, our Chief Financial Officer; Mark Shearman, our Chief Scientific Officer; Theresa Heah, our Chief Medical Officer; Matt Feinsod, our Executive Vice President of Global Strategy and Development; and David Knop, our Executive Director of Process Development.

While this is a larger group than we've had on previous calls, I've asked the broader team to join us in order to share with you a wealth of new data and information that we believe validates our technology platform. I'm certain you'll find our expertise and insights helpful.

We're excited to share with you today new data that includes the planned top line 6-month XLRP dose escalation data as well as preliminary 3-month data from the XLRP dose expansion cohort showing stability of visual function in the periphery and improvement of visual function centrally. We are also pleased to provide an early look at data indicating biologic activity in our achromatopsia trials. Much of this information is included in the slide deck accompanying our prepared remarks, which will be available on the AGTC website following this call.

We believe these data across 3 clinical indications and the company's extensive preclinical data and manufacturing excellence enhance and strengthen AGTC's leadership in the ophthalmology gene therapy arena.

We've made great progress over the last 12 months across our clinical programs in XLRP and achromatopsia, and we are leveraging our expertise in AAV gene therapy to expand our development activities into additional disease areas.

Later in the call, Mark will discuss the progress we've made in our preclinical programs, all of which take advantage of our existing expertise, knowledge, resources and infrastructure in order to maximize our likelihood of success while streamlining the development progress.

In the fourth quarter, we made several strategic management enhancements in anticipation of late-stage clinical development and commercialization activities shown in Slide 4. These include appointing Theresa Heah as our new CMO. Theresa joins us with more than 10 years of senior executive pharmaceutical experience, including successfully overseeing late-stage drug development and commercialization, and it will help her to guide our ongoing clinical programs through the clinical and regulatory landscape toward approval, and we look forward to her valuable contributions.

With Theresa's appointment as CMO, Matt Feinsod has transitioned to Executive Vice President of Global Clinical Strategy and Development. We also appointed Brian Krex as General Counsel. Brian has extensive commercialization experience, most recently having served as Vice President and Global Head of Commercial and Regulatory Law with Alexion Pharmaceuticals. We believe this added expertise will enhance our ability to successfully develop and commercialize our innovative product pipeline. We are confident we have the right executive team in place to advance our company through late-stage development and eventual regulatory approval.

I will now turn the call over to Theresa who will provide our clinical updates.

Theresa Heah - *Applied Genetic Technologies Corporation - Chief Medical Officer*

Thank you, Sue. We're excited to provide the planned XLRP top line 6-month data from the dose escalation groups as well as a preliminary look at 3-month data from the XLRP dose expansion group, showing a favorable safety profile with stability of visual function in the peripherally dosed patients and improvement of visual function in 50% of centrally dosed patients.

We are also providing an early look at data from the achromatopsia B3 and A3 trials showing signs of clinically meaningful improvements in light discomfort at 3 months.

Taken together, we believe this data provides solid rationale for the advancement of our clinical programs in our efforts to bring important new products to patients in need.

I will start with an update for our XLRP program and a brief disease overview. As shown on Slide 7, patients with XLRP do not produce the RPGR protein, which leads to poor photoreceptor function and retinal degeneration. This results in early night blindness and progressive constriction of visual fields. Typically, XLRP patients are legally blind by age 45.

Slide 7 also shows patients with XLRP see the world through constricted visual fields. As a reminder, this study used a subretinal injection as the route of administration, and patients were injected centrally or peripherally in order to provide maximum benefit on each patient's baseline characteristics. There are currently no effective therapies for XLRP.

Slide 8. AGTC's product candidate is designed to provide a functional copy of the RPGR gene to photoreceptors delivered by a novel engineered AAV capsid as we believe it could transform the care, outcomes and quality of life for patients living with this disease.

The dose groups I'll be discussing today are summarized in Slide 9, and include 10 patients who have completed 6 months follow-up and 7 patients who have completed 3-month follow-up. In addition to the 17 patients we will be discussing, we have dosed an additional 5 patients for a total of 22 patients dosed to date. We will continue dosing additional patients under the Phase I/II protocol to further enrich our analysis and build a robust set of safety and efficacy data to support our biologics license application, or BLA, filing. We believe this approach will maximize the benefit to the greatest range of patients, putting us in a solid position for potential approval and commercialization.

Before I review the clinical data, I would like to note that we have shared this information with a group of retinal experts who have extensive expertise in treating XLRP and they concur that the data, if sustained, would mark an inflection point in the treatment of these diseases.

Slide 10 outlines the patient demographics we will discuss today. For each patient, we chose to dose the eye with the lower visual function.

As described on Slide 11, the data continue to demonstrate a favorable safety profile across the 17 patients treated for our XLRP candidate. Importantly, no dose-limiting inflammatory responses have been observed in patients treated to date, and we've had no secondary inflammatory response requiring readministration of any steroids. Based on the safety and tolerability, our Data Safety Monitoring Community -- Committee, DSMC, supported continued dose escalation in the trial as well as dosing of pediatric patients.

Slide 13. Let me begin with a 6-month data from the patients dosed peripherally. 8 patients from the dose escalation portion of the study were dosed peripherally. Peripherally dosed patients showed stable visual function at 6 months as measured by visual fields and visual acuity. These encouraging data in the periphery is unique to AGTC.

Slide 14 shows stable visual sensitivity, both for all patients combined and by dose group.

Slide 15 shows the stable best corrected visual acuity, or BCVA, both for all patients combined and by dose group.

Slide 16. While patients will need to be followed for additional time for -- to truly show stabilization in this degenerative disease, based on the data to date, we can conclude that our XLRP candidate is generally well tolerated and leads to stable visual function at 6 months. This maintenance of peripheral vision is an important milestone for these patients.

Let me turn to the 3-month data from the centrally dosed patients. Moving onto Slide 18. We are excited that preliminary data from 9 patients who have reached at least 3-month time point dosed centrally shows evidence of improvement in visual function. We have seen measurable improvements in visual sensitivity for 4 of 8 patients, 1 at the middle dose and 7 at the high dose, a response rate of 50%. We define a responder as a patient who has improvement in visual sensitivity within the treatment area that is beyond the testing variability of at least 2 different test dates. Note that for 1 of the 9 centrally dosed patients, we do not have a complete data set for sensitivity measurements.

Moving to Slide 19. Another way to analyze improvements in visual sensitivity is by identifying the percentage of patients who have greater than 5 loci in the treated area with at least a 7 decibel increase compared to baseline. We have seen 38% of patients meet this cut-off at month 3. For context, the best reported result seen to date was 33%.

As shown on Slide 20, all 9 patients treated centrally also had stable or improving visual acuity, a traditional hallmark of visual function, results that have not been disclosed in other XLRP clinical trials to date.

Moving to Slide 21. We can see that Optical Coherence Tomography, or OCT, analysis for all 9 patients had no measurable decline in photoreceptor structure in either the treated or untreated eye. We believe that all these outcomes taken together strongly suggest that our product candidate could provide significant clinical benefit to patients.

Now let's take a more detailed look at the data from of -- from the 4 identified responders. Slide 22 shows the mean change in sensitivity from baseline within the bleb at months 1, 2 and 3 compared to the untreated eye. This data compared favorably to what has been reported from others, which are also shown on the slide.

Slide 23 shows an example of microperimetry results within the treated area as a heat map for the mid-dose responder, indicating meaningful improvements in visual sensitivity compared to the untreated eye and compared to baseline. Increases in visual sensitivity can provide vision under lower light conditions, sharper vision and/or a wider field of vision. And this patient shows both wider fields of vision and brighter fields of vision.

Similarly, Slide 24 shows microperimetry results for the 3 high-dose responders. As you can see, all 3 show meaningful improvements in visual sensitivity compared to the untreated eyes and to baseline, all showing brighter vision and 1 also showing wider fields of vision.

To reiterate, stabilization of peripheral vision and improvement in central vision is important and clinically meaningful because reductions in visual sensitivity are a key hallmark of this degenerative disease and negatively impact patients' ability to fully engage in daily living and work activities. Importantly, anecdotal statements from the patients support our analysis of the measurable improvements as being meaningful to their daily lives. And many of these statements are captured here on Slide 25. For example, one patient said, "Objects appear brighter."

Slide 26. The biologic activity observed to date in this clinical trial, assuming is sustained through the 12-month time point, reinforces our confidence that the trial design will yield the data we need to advance our XLRP program to a pivotal trial. We believe stabilization of decline in the peripheral vision in combination with improvement in central vision with a favorable safety profile will be of great importance to patients.

Our current expectation is that we will be communicating with the FDA on the data to date and the proposed design of the pivotal trial over the next several months. Our goal is to advance our discussions with the FDA such that, at the end of Phase II meeting, we will come to a definitive conclusion on the design of the pivotal trial, allowing us to initiate the trial in 2020. We have also initiated all activities to support manufacture of the pivotal clinical trial material in Q1 of 2020.

Slide 27 summarizes our current thoughts on the next steps in product development. We expect the trial to include 2 dose groups with an active phase of 12 months and that the most likely endpoint will be microperimetry supported by continued safety.

Now let me turn to our Phase I/II clinical trials in achromatopsia, and again begin with a brief overview of the disease. As shown in Slide 28, patients with achromatopsia either do not produce the CNGB3 or do not produce the CNGA3 protein, which leads to a lack of function in cone photoreceptors. As a result, achromatopsia patients have extremely poor vision and are legally blind. They also have extreme light discomfort, which makes them day blind, and have a complete loss of color discrimination.

AGTC's programs are designed to provide functional copies of the CNGB3 or CNGA3 gene depending on the patient-specific mutation to cone photoreceptors delivered by an engineered AAV capsid and a cone specific promoter. Here again, we believe that our treatment approach has the potential to transform the lives of patients with achromatopsia who currently have no effective therapies.

Today, we are pleased to report early data from both ongoing achromatopsia trials. As with XLRP, the safety profile in both trials remain favorable. The DSMC has supported continued dose escalation and dosing of pediatric patients. To date, we have enrolled 15 patients in the B3 trial and 12 patients in the A3 trial. Today, we'll be discussing data from a subset of those patients, and the dose groups I'll be discussing are summarized in Slide 30.

Light discomfort is a significant issue for achromatopsia patients, and we have developed a quantitative measurement with our academic collaborators. Slide 31 provides a visual depiction of how loglux values, the output of the measurement, correspond to typical illumination levels and demonstrate how is 1 loglux improvement in light discomfort is clinically meaningful to patients.

Slides 32 and 33 summarize the early biologic activity results from both achromatopsia trials. Light discomfort measurement quantifies the highest light level tolerated by patients. As shown on Slide 32, 1 patient in the mid-dose group and 2 patients in the high-dose groups of the B3 trial have had clinically meaningful improvements defined as greater than 1 log change from baseline in light discomfort at 3 months.

As shown in Slide 33, 1 patient in the mid-dose level in the A3 trial has shown clinically meaningful improvement using the same definition. Importantly, anecdotal statements from the patients shown on Slide 34 support these improvements as being meaningful to their daily lives. For example, 1 patient has said, his headaches from eyestrain have stopped.

Slide 35. As with XLRP, this evidence of biological activity furthers our confidence in the design of these trials and the potential to advance both achromatopsia programs to pivotal trials. As we analyze the data, we envision we will continue to see this type of meaningful improvement in light discomfort as well as other measures of visual function.

We believe the collective encouraging data for all 3 of our ongoing clinical trials is supportive of our product design, construct and manufacturing process and highlights our technical excellence and is reflective of the team's execution over the past year.

I will now turn the call over to Mark, who will briefly review our research efforts and new preclinical program priorities.

Mark S. Shearman - *Applied Genetic Technologies Corporation - Chief Scientific Officer*

Thank you, Theresa. Slide 37. As we have previously disclosed, over the past year, we've conducted a strategic review of our preclinical pipeline. We believe that each of these programs have significant technical, clinical and commercial potential and represent attractive market opportunities in which gene therapy can potentially change the treatment paradigm.

I want to emphasize Sue's point that all of them build on the very strong foundation in vector design, manufacturing, preclinical development and clinical trial design that we have established in the course of conducting our current clinical trials.

We are excited to report that we are expanding our ophthalmology portfolio with 2 new programs. The first targets the dry form of age-related macular degeneration, AMD, which represents a compellingly large market opportunity given that the disease affects over 24 million people globally. This program leverages our existing expertise in ophthalmic gene delivery and large-scale manufacturing, while providing another opportunity to expand our pipeline beyond diseases that solely result from mutations in single genes. We are currently finalizing proof-of-concept animal work prior to initiating IND-enabling safety studies.

The other new ophthalmology program targets an orphan indication with a substantial patient population, leveraging our expertise and capabilities in the orphan ophthalmology space. In the 2 ocular programs, we have reengineered the DNA cassette to fit into AAV vectors while still producing a fully active protein.

In addition to our ongoing research in otology indications, we are expanding our strategic focus into central nervous system, or CNS, disorders. Our first effort in the CNS is adrenoleukodystrophy, or ALD, which is an X-linked genetic disease that affects approximately 10,000 people. Preclinical data support a gene therapy-based approach to treating the disease and warrant advancing it into our pipeline. We are currently conducting a final targeting study in nonhuman primates before moving into IND-enabling safety studies.

We are also working on 2 additional rare genetic CNS indications that have substantial patient populations and well-defined clinical phenotypes. We are using novel technologies to design the gene cassette and administer the product to the optimal location. We believe these programs play to our strengths in large-scale manufacture of AAV vectors, while also providing us with the opportunity to expand our base of experience into AAV delivery to the brain and spinal cord. We are currently conducting construct optimization, nonhuman primate targeting and proof-of-concept animal work and recently had the first full meeting of our CNS Advisory Board that includes researchers, clinicians and surgeons. In the CNS programs, we are using our robust vector targeting and design capabilities to specifically address the unique pathologies of the disease we are targeting, in one case using a unique trivalent gene cassette.

Slide 38. Over the past year, we have also made significant progress with our manufacturing capabilities. Our team has made great advances in our efforts to build a best-in-class manufacturing process. As shown on the slide, the team has successfully developed processes that provide a tenfold increase in productivity compared with traditional AAV manufacturing methods. This enhanced process can be scaled to meet expanding demand, including for indications that require large doses or represent large patient populations and is supported by a comprehensive asset development program to ensure that manufacturing is not a rate-limiting step as we approach the filing of a BLA.

On Slide 39, I'd like to briefly discuss several sets of preclinical studies in nonhuman primates. This research continues our industry-leading efforts to deeply understand the underlying technology and develop a breadth of data to support potential product approval and commercialization. We have completed studies that have identified several factors that influence the ocular immune response, showing that these responses appear to be dose dependent, not linked to specific AAV capsid type or manufacturing method and have not affected by preexisting immunity to AAV. We have also been exploring the safety and efficacy effects of administering multiple subretinal blebs, which to date has been successfully achieved in nonhuman primates. And lastly, we are examining the effects of redosing vector and its potential to enhance the therapeutic option for patients.

The ongoing robust preclinical analysis and the advancement of our manufacturing process demonstrates our commitment to conducting innovative drug development in a way that ensures we will have attained an optimized target product profile when we reach commercialization.

I will now turn the call over to Bill, who will briefly review our fiscal results for the fourth quarter and full 2019 fiscal year.

William A. Sullivan - *Applied Genetic Technologies Corporation - CFO*

Thank you, Mark. Our fourth quarter and full year 2019 financials were included in our press release, which was distributed a short while ago, and Slide 41 provides an overview of our financial results.

For fiscal year 2019, we generated a net loss of $2.0 million compared to a net loss of $21.3 million for fiscal year 2018. The decrease in net loss was primarily due to a $17.5 million increase in revenues and a $1.5 million decrease in G&A expenses, which was partially offset by a $1.0 million increase in R&D expenses. The $17.5 million increase in revenues is primarily due to recognizing revenue of $20.4 million associated with the termination of the collaboration agreement with Biogen effective March 8, 2019. Going forward, no additional collaboration revenue related to the Biogen agreement will be recognized. The $1 million increase in R&D expenses is primarily due to incurring sublicense expense of $2.3 million associated with receiving a milestone payment from Biogen and increased employee-related costs partially offset by decreased preclinical R&D spending. The $1.5 million decrease in G&A expenses was primarily driven by decreased employee-related and share-based compensation expenses.

Now move onto our financial guidance. We ended fiscal year 2019 with a strong balance sheet. Total cash, cash equivalents and investments as of June 30, 2019, were $82 million. We believe these funds will be sufficient to allow AGTC to generate data from our ongoing clinical programs, move our preclinical optogenetics program in collaboration with Bionic Sight into the clinic, initiate activities to ensure efficient transition into pivotal trials and fund the prioritized preclinical programs discussed today into the first half of 2021. We expect that total cash, cash equivalents and investments as of June 30, 2020, to be between $30 million and $40 million.

That concludes the team's remarks today. Operator, you may now open the call to question-and-answer period.

QUESTIONS AND ANSWERS

Operator

(Operator Instructions) Our first question comes from the line of Joe Pantginis with -- from H.C. Wainwright.

Joseph Pantginis - *H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst*

Congratulations on the data, very nice to see these early data. So a couple of things, if you don't mind. So when you look at the Slides 14 to 15 for stable visual sensitivity, I just want to make sure I get some color on the interpretation of the data. Are the -- say, like on Slide 14, for example, for peripherally dosed patients, the bars that are approximately 6 to 8, the ones that go horizontally, are those the bounds you were talking about with regard to all of the pretesting that was conducted that essentially is what you would consider the noise?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Joe, that's a really good question, and thank you for attending the call today. The bars there represent the variability in the patient data. So patients start with different baselines. And so when you kind of smoosh those together, that's what the arrow bars represent. So the arrow bars are patient differences, not the repeatability coefficient that we were discussing before.

Joseph Pantginis - *H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst*

And I'm just talking about like the horizontal lines, not the deviation bars.

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

I'll let Theresa go into some more detail.

Theresa Heah - *Applied Genetic Technologies Corporation - Chief Medical Officer*

So in the graph, you can see the dotted line is stated in the legend, dotted lines equal baselines. So those are in reference to baselines for the treated eyes and also to the untreated eyes.

Joseph Pantginis - *H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst*

Okay. No, I understand. And then -- so when you have the baselines that were created, I guess when you look at now, I guess a comment about the natural history because being able to see stabilization as you mentioned, I mean this is very powerful data in my opinion and hopefully it should be received such as well. What you would expect to see even at month 3 for some of the deterioration in XLRP patients?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So that's another good question. XLRP patients actually do lose their peripheral vision first. So some kind of stability in the periphery is very important to patients. Theresa did mention that 6 months is a short period of time to really make a definitive call. And so that's why we'll be continuing to follow these patients over the next several months to see if that stability is maintained.

Joseph Pantginis - *H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst*

No, that's really helpful. And my last question, and thanks for your patience, I guess, is just talking about as the studies move forward and going into pivotal studies, any further optimization of delivery that you might be looking at or changes to the procedure? And what I'm getting at, for example, is like, for example, patients that don't necessarily respond, do you believe that there is any potential, say, vector that sort of spills out from the injection side of the bleb that maybe might reduce efficacy?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So that's a very good question, and I'm going to let Theresa answer that.

Theresa Heah - *Applied Genetic Technologies Corporation - Chief Medical Officer*

These are subretinal injections, and these are cell-based proteins that do not spread throughout the retina. So the treatment affects really in the area of the bleb. So in terms of lessons learned throughout, we've enhanced our surgical training. Our surgeons are very well trained in terms of subretinal injections.

Operator

Our next question comes from the line of Matthew Luchini with BMO Capital Markets.

Matthew W. Luchini - *BMO Capital Markets Equity Research - Analyst*

Great. Congrats on the progress. So I guess I have kind of one overarching question and with it some subparts. And what that question is, is it seems like this is the 6 months data, there are 10 evaluable patients and there is some initial discussion of the stabilization of those that were treated peripherally at 6 months. But I don't see anything about the 2 patients that were centrally dosed post 3 months. So I guess to that point, I'd like to know a little bit more about what happened to those 2 patient between months 3 and 6. Maybe being a little bit more specific, could you talk a little bit about where they included in the 50% responders that you highlighted at week 12? And if so, maybe what happened between months 3 and 6, and if not, why not? Similarly, sort of the same question you mentioned the 7 decibel gain patients. Were any of the -- were either of those 2 patients in that group? And then lastly, the 1 patient that was nonevaluable, I thought it was from microperimetry. Could you just provide a little bit more color about what happened there? And then maybe I'll get back in the queue after those.

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So this is Sue, Matt, and thank you for those questions. And having 6 questions in one question, so hopefully we can -- we will walk through them all, and please jump in and remind us if we forget something. So there were 2 patients from the dose escalation that were dosed centrally. Those 2 patients from the dose escalation dosed centrally are included in the count of 9 centrally dosed patients. So we have provided in our analysis, and those patients are included in the analysis that gets us to that 50% response rate, that gets us to the 4 responders. So the 4 responders include 1 dose escalation patient and 3 dose expansion patients. The patient that we don't have full data on is one of the dose escalation patients because we don't have the microperimetry, but that patient is included in the visual acuity analysis. We only included in the analysis 3-month data from those 2 patients because that's the only data that we had for all 9 patients together. So we wanted to be comparing apples-to-apples, and we're now providing the 6-month data on those patients, so we have 6 months data on all of the patients.

Matthew W. Luchini - *BMO Capital Markets Equity Research - Analyst*

Okay. But -- so just to confirm, you're saying that 1 of the 4 patients that constitute your 50% responders includes one of the patients that has 6 months worth of follow-up? Is that also true for the patients with the 7 decibel gain?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Yes.

Operator

Our next question comes from the line of Jim Birchenough with Wells Fargo.

James William Birchenough - *Wells Fargo Securities, LLC, Research Division - MD and Senior Biotechnology Analyst*

Just want to add my congratulations for the data and the promising early results. So maybe just high-level question. You kind of alluded to it in your comments, but how would you contrast the data that you reported today versus what we've seen from Nightstar. I think you alluded to it, but I'll ask specifically. When you look at their results and your results, how do you position your results against what we saw from them?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So I think -- Jim, thank you for the question, and thank you for dialing in. I think that there is 3 things that we want to talk about. First of all, we do think our data is stronger and more robust and broader. We have a broader set of data. On specifically visual sensitivity, we think we're very comparable. On visual acuity, we are able to present some data that we think is very encouraging that has not been reported by others. And lastly,

we think that the peripheral data showing stability is something no one else has been presenting, and we think this is very important to patients because, of course, that's where they lose their vision first, is in the periphery. So the breadth of data we have, we think, is very strong.

James William Birchenough - *Wells Fargo Securities, LLC, Research Division - MD and Senior Biotechnology Analyst*

And, Sue, just in terms of that peripheral benefit in stabilizing visual fields, is it too early to make comparisons to untreated areas or comparable areas that are untreated? And would you expect to make those comparisons at 12 months?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So we do think it is very early. We think the data is very encouraging, as Theresa pointed out, but it is early to show real stability over time compared to the untreated eye. And so that's why we will be continuing to follow these patients out through 12 and 18 months and, actually, the peripherally dosed patients are in active follow-up through 2 years.

James William Birchenough - *Wells Fargo Securities, LLC, Research Division - MD and Senior Biotechnology Analyst*

And then maybe just one final question and clarification. I thought I heard Theresa mention when considering parameters of potential pivotal study that microperimetry would be used. So is that to say the main focus will be on central vision? And I guess the second part of it is, when you have a profile that shows both stability of the periphery and improvement centrally, could you envision a paradigm where you dose in both locations?

Theresa Heah - *Applied Genetic Technologies Corporation - Chief Medical Officer*

So we have data now at 3 months showing improvement centrally. Hence, our pivotal trial will be based on the microperimetry as the primary endpoint. As we continue to look at longer time points on the peripherally dosed patients, we will enhance our clinical program to include peripherally dosed patients in the later stage of the pivotal trial.

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

And I also think that -- sorry for the train noise. I also think that it is important to note Mark's comments about all of the nonhuman primate work that we're done -- we're doing that we are developing the data set to show that multiple blebs in the same eye are appropriate and that redosing into the eyes is appropriate. So again, that shows that we're thinking about long-term development and developing that complete set of data necessary to move forward.

James William Birchenough - *Wells Fargo Securities, LLC, Research Division - MD and Senior Biotechnology Analyst*

I'm going to ask one more question, I promised not to, but I have to ask, when you consider focus on improvements in vision, that's usually easier to say that's a divergence from natural history. And so given that, do you think the pivotal study could get away without a control group? Or how would you see the control of your pivotal study?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

I think that the guidance from the FDA has been pretty clear on what they like pivotal studies to look like in that they like to see 2 arms in the pivotal study to be able to show a dose differentiation and to be able to randomize patients and eliminate bias.

Operator

Our next question comes from the line of David Nierengarten with Wedbush Securities.

David Matthew Nierengarten - *Wedbush Securities Inc., Research Division - MD & Head of Healthcare of Equity Research*

Maybe to follow up a little bit on the prior questions. I guess when or how would you communicate a dosing to focus on the central part of the [IRP] patients or to the peripheral or both peripheral and central portions? And then an additional question would be, given the lack of inflammation that we see -- or that you've seen in the study, is it possible to go up to another dose level? Or is that contributing to your confidence in perhaps being able to dose in 2 different areas in the eye?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Thank you for those questions, David. I think that, as Theresa stated, we're focusing on the microperimetry right now because we have seen this improvement in vision in the centrally dosed patients and that is more straightforward, as a couple of you pointed out. So that would be our initial focus. But we are definitely focused on continuing to develop the data in the periphery, so we could add that into clinical development as we move forward. And your last dose escalation question is about that always in a Phase I/II trial, you always have the opportunity to amend your protocol and expand your dosing as long as you continue to see safety. I think what we're most excited about, about the lack of inflammation, is that you say that much better for the patients in the long term and gives you a lot of optionality to be able to dose in multiple locations, et cetera.

David Matthew Nierengarten - *Wedbush Securities Inc., Research Division - MD & Head of Healthcare of Equity Research*

And maybe just a quick follow-up to confirm or to ask. Were there -- or remind us, were there any steroid protocols or anti-inflammatories agents used in the study?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Yes, all of our retinal programs, whether it's XLRP or achromatopsia, all have a standard steroid regimen.

Operator

(Operator Instructions) Our next question comes from the line of Yun Zhong from Janney.

Yun Zhong - *Janney Montgomery Scott LLC, Research Division - Equity Research Analyst & Director of Biotechnology Research*

So first a follow-up on the peripheral dosing versus central dosing. And it looks like the most recent 7 patients in the dose expansion cohort all received central administration. So I wonder is that because you saw the kind of encouraging data from the 2 patients from the previous phase who got central dosing that you decided to dose all patients in the central region? Or was that the original design that you were going to dose all patients in the -- or -- in the central region of the retina?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So thank you for the question, Yun. We had always planned to dose patients according to what was best for the particular patient. I think as we develop -- as the patient -- as the data was developing and we saw more encouraging improvements, even though the stabilization is very important to these patients in the periphery, when we saw the improvements in the centrally dosed patients, both in cohort 2 and in the higher dose, we did shift such that we dose more of the patients, we looked for more patients that we could dose centrally in the expansion cohort.

Yun Zhong - *Janney Montgomery Scott LLC, Research Division - Equity Research Analyst & Director of Biotechnology Research*

Okay, I see. And on the improvement in the visual acuity, I wonder do you -- are you able to share how many letters or is it close to the 15 letters that are typically required to show clinical -- meaningful clinical benefit?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So we do have the letters on the slide of what the improvement was. I think that 15 letters is really a standard establish for large market products such as AMD. And I'll let Theresa go through that data in a little bit more detail.

Theresa Heah - *Applied Genetic Technologies Corporation - Chief Medical Officer*

So to your question, on Slide 20, that's the arrow bars that show you the minimum and maximum visual acuity improvement. We saw patients up to close to 2 lines improvement.

Yun Zhong - *Janney Montgomery Scott LLC, Research Division - Equity Research Analyst & Director of Biotechnology Research*

I see. Okay. And the last question. I believe the study included some patient-reported outcome as the efficacy measure. Are you able to provide any information? Or is that still premature?

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

So we haven't fully analyzed the actual patient-reported outcome survey, but we did include in our slides today many of the patient-reported anecdotal comments that were quite supportive of the quantitative improvements.

Operator

Since there are no further questions left in the queue, I would like to turn the call back over to Sue Washer for any closing remarks.

Susan B. Washer - *Applied Genetic Technologies Corporation - President, CEO & Director*

Well, thank you all for joining us today on the call. I believe that the data presented position us for leadership in the field of gene therapy. The preliminary data from these trials show improvements in key endpoints and are very promising. As Theresa noted earlier, we have shared this information with a group of retinal experts who have extensive expertise in treating these diseases, and they concur that, if sustained, these results would mark an inflection point in the treatment of these diseases. We look forward to sharing the top line 6-month data from the XLRP expansion cohort and the top line 6-month data from the achromatopsia dose escalation cohorts with you later this year.

We are already engaged in multiple activities that will allow advancement to pivotal trials as quickly as possible, and we expect to move forward to pivotal trials in 2020 once we have final agreement with the FDA on trial design. We will also continue additional dosing in all 3 trials under the current protocol to enrich and build a robust data set to support our BLA filing, thus maximizing the benefit to the greatest range of patients and create the strongest package for approval and commercialization.

We have great confidence in our manufacturing capabilities to support all our preclinical programs as well as the clinical programs and have in place the resources and infrastructure we need to successfully advance to pivotal trials and approval. As I always do, I will close today's call by thanking the patients, physicians and the AGTC team for their dedication to our cause and their support of our efforts to transform the treatment for rare ophthalmic, otologic and CNS diseases. I look forward to sharing our joint achievements with you in the months ahead.

Operator

This concludes today's teleconference. You may now disconnect your lines at this time. Thank you for your participation, and have a wonderful day.