THOMSON REUTERS STREETEVENTS

EDITED TRANSCRIPT

EDIT - Q4 2019 Editas Medicine Inc Earnings Call

EVENT DATE/TIME: FEBRUARY 26, 2020 / 10:00PM GMT

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

CORPORATE PARTICIPANTS

Charles Albright *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Cynthia L. Collins *Editas Medicine, Inc. - President, CEO & Director*

Judith R. Abrams *Editas Medicine, Inc. - Chief Medical Officer*

Mark J. Mullikin *Editas Medicine, Inc. - Senior Director of Finance & IR*

Michelle Robertson *Editas Medicine, Inc. - CFO, Principal Accounting Officer, Treasurer & Assistant Secretary*

CONFERENCE CALL PARTICIPANTS

Amanda Louise Murphy *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

Geulah Livshits *Chardan Capital Markets, LLC, Research Division - Senior Research Analyst*

Joseph John-Charles Thome *Cowen and Company, LLC, Research Division - VP of Healthcare*

Silvan Can Tuerkcan *Oppenheimer & Co. Inc., Research Division - Associate*

Steven James Seedhouse *Raymond James & Associates, Inc., Research Division - Research Analyst*

Thomas Francis Lavery *Morgan Stanley, Research Division - Research Associate*

Yanan Zhu *Wells Fargo Securities, LLC, Research Division - Associate Analyst*

PRESENTATION

Operator

Good afternoon, and welcome to Editas Medicine's Fourth Quarter and Full year 2019 Conference call. (Operator Instructions) Please be advised that this call is being recorded at the company's request. I would now like to turn the call over to Mark Mullikin, Vice President of Finance and Investor Relations at Editas Medicine.

Mark J. Mullikin - *Editas Medicine, Inc. - Senior Director of Finance & IR*

Thank you, operator. Good afternoon, everyone, and welcome to our fourth quarter and full year 2019 conference call. Shortly after the market closed, we issued a press release providing our financial results and corporate updates for the fourth quarter and full year 2019. A replay of today's call will be available on the Investors and Media section of our website approximately 2 hours after its completion. After our prepared remarks, we will open the call for Q&A.

As a reminder, various remarks that we make during this call about the company's future expectations, plans and prospects constitute forward-looking statements for purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995.

Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including those discussed in the Risk Factors section of our most recent annual report on Form 10-K, which is on file with the SEC. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing our views as of any subsequent date.

Except as required by law, we specifically disclaim any obligation to update or revise any forward-looking statements, even if our views change.

Now I will turn the call over to our Chief Executive Officer, Cindy Collins.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

Thank you, Mark. Good afternoon, and thank you, everyone, for joining us for our corporate update call for the fourth quarter and full year 2019.

In addition to Mark, I'm joined by several members of the Editas executive team, including: Judith Abrams, our Chief Medical Officer; Charlie Albright, our Chief Scientific Officer; Michelle Robertson, our new Chief Financial Officer; and Tim Hunt, our Senior Vice President of Corporate Affairs. We are at an exciting point at Editas Medicine, developing differentiated transformational medicines across a range of serious diseases and nearing the point of seeing them work in patients.

On today's call, we will review some of what we have achieved over the past year, then look ahead to 2020 and beyond.

So let's start with some of our accomplishments over the past year. We initiated the first ever clinical trial of an in vivo CRISPR medicine in collaboration with Allergan of EDIT-101 for patients with LCA10. We started IND-enabling studies for EDIT-301 as a potentially best-in-class medicine for the treatment of sickle cell disease. We amended our collaboration with Celgene, now part of Bristol-Myers Squibb to focus on developing engineered alpha-beta T cell medicines for cancer. We are excited to continue our work with the leader in treating blood cancers.

With the amendment, we received a $70 million cash payment and regained rights to develop engineered gamma T cell medicines in oncology. We accelerated our efforts to develop engineered allogeneic NK cell medicines for cancer through a newly formed research collaborations. We enabled our development of healthy donor NK cell medicines for solid tumors with cell expansion technology from Sandhill Therapeutics, and we are advancing iPSC-derived NK cell medicines using technology from Bluerock Therapeutics. And we added outstanding executive leadership with the appointment of Judith Abrams as our Chief Medical Officer; Michelle Robertson as our Chief Financial Officer and Harry Gill as our Senior Vice President of Operations. I am pleased to have Judith and Michelle joined me on the call today.

These accomplishments give us momentum into the coming year. In 2020, we plan to announce dosing of patients in the first quarter in the Brilliance Phase I/II trial of EDIT-101 in collaboration with our partner, Allergan. We plan to nominate a development candidate for autosomal dominant retinitis pigmentosa 4 or RP4, file an IND for EDIT-301 for the treatment of sickle cell disease, initiate IND-enabling studies for an allogeneic, healthy donor NK cell medicine candidate to treat solid tumors, and finally, present in vivo preclinical proof-of-concept data for an engineered iPSC-derived NK cell medicine to treat solid tumors.

Now let me turn the call over to our Chief Medical Officer, Judith Abrams, to update you on our Phase I/II clinical trial of EDIT-101.

Judith R. Abrams - *Editas Medicine, Inc. - Chief Medical Officer*

Thanks, Cindy. It's my pleasure to join all of you on the call today. As Cindy mentioned, last year, we opened the Brilliance Phase I/II study for patients with LCA10 for enrollment. Enrollment activity has accelerated in recent months and we expect the announcement on the first patient dosing in the first quarter of this year. As a reminder, the first patient dose in the Brilliance clinical trial will mark a significant milestone towards delivering on the promise potential of CRISPR medicines to durably treat devastating diseases such as LCA10. I'm also pleased to share that as our research pipeline continues to regress to the clinic, we are building out our senior leadership in our clinical organization.

I'll now turn over the call to our Chief Scientific Officer, Charlie Albright, to discuss our broader pipeline.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Thank you, Judith, and thank you all for joining us on the call. Following EDIT-101, our next in vivo ocular program is EDIT-102 for treatment of Usher Syndrome 2A or USH2A.

Like LCA10, USH2A is an inherited retinal disease that affects photoreceptors and leads to blindness. At the product level, EDIT-102 is nearly identical to EDIT-101, in that EDIT-102 uses the same AAV5 delivery vector, proprietary Staph aureus Cas9 enzyme in photoreceptor-specific promoter as does EDIT-101.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Preclinical studies support the advancement of EDIT-102 into IND-enabling study since we have demonstrated editing levels, mRNA transcriptional levels and phenotypic restoration that are consistent with therapeutic benefit. Based on these data, we delivered a data package for EDIT-102 to Allergan for potential licensing and development as part of our strategic alliance we formed in 2017.

Our learnings with EDIT-101 and EDIT-102 are being leveraged for other in vivo editing medicines, in particular, AAV delivery of Staph aureus Cas9 is used in our medicine aimed at RP4, another inherited retinal disease, where we plan to declare a development candidate for IND-enabling studies later this year.

Finally, we've expanded our in vivo research pipeline into neurologic diseases in collaboration with AskBio and hope to present initial data this year.

Transitioning to our engineered cell medicines programs, we are developing at EDIT-301 as a potential best-in-class medicine for sickle cell disease and beta thalassemia. Our program uses the Cas12 enzyme to edit the HPG I/II promoter in the beta-globin locus to induce fetal hemoglobin in hematopoietic stem cells. Cas12a is proprietary to Editas and previously known the Cpf1. For those unfamiliar with our program, our approach is differentiated from competitors who either edit the BCL11A enhancer locus or use gene therapy. We shared our latest data at the American Society of Hematology Conference in December.

EDIT-301 added to the degenomic region where human mutations are found to increase fetal hemoglobin. This genetic support is important as these data reduce the risk with human efficacy and safety.

In contrast, the BCL11A enhancer approach does not have human genetic validation.

For EDIT-301, preclinical data shows that HPG editing and hematopoietic stem cells is durable, induces high levels of fetal hemoglobin and does not negatively impact blood cell lineage. We plan for EDIT-301 IND filing for the treatment of patients with sickle cell disease by year-end.

The other major focus for our engineered cell medicines programs is to treat cancer. We're developing an engineered alpha-beta T cell medicines for cancer in collaboration with Bristol-Myers Squibb, the leader in treating blood cancers where we believe alpha-beta T cell medicines have the potential to be particularly effective.

In addition, we are advancing our wholly-owned programs by editing innate immune cells, including NK and gamma delta T cells to treat solid tumors. We plan to begin IND-enabling studies midyear for an edited healthy donor NK cell medicine to treat solid tumors.

We recently announced work with Sandhill Therapeutics to accelerate our healthy donor medicines. Sandhill brings established processes, manufacturing infrastructure and proprietary expansion method.

In parallel with the healthy donor program, we are advancing engineered iPSC-derived NK cells or iNK cells as medicines for solid tumors.

In partnership with BlueRock Therapeutics now part of Bayer, we've made great progress editing and differentiating iPSC cells to form iNK cells. Combining the technologies of iPSCs and CRISPR gene editing brings together 2 platforms that can revolutionize engineered cell therapies.

We're excited about this potential and look forward to updating you on our progress in the near future.

Now I'll turn the call over to our newly appointed Chief Financial Officer, Michelle Robertson.

Michelle Robertson - *Editas Medicine, Inc. - CFO, Principal Accounting Officer, Treasurer & Assistant Secretary*

Thanks, Charlie. I'm pleased to join you all today to introduce myself and present the company's latest financial results. I've being working in finance in the biotech industry for more than 25 years, most recently as the CFO of Momenta Pharmaceuticals, and prior to that, in a number of leadership roles at Genzyme, Baxalta and Ironwood. For me, what differentiated Editas from other companies is the infinite possibilities to develop medicines

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

that we are working on to help patients, and high-pressed to think of another company with as much potential as Editas to develop truly transformative medicines for patients with diseases of unmet need. In this role, the opportunity to make a difference in the future of the company was a big drawer.

Now turning to the numbers. We have summarized our financial results for the fourth quarter and full year 2019 in the press release that we issued earlier today.

Our cash, cash equivalents and marketable securities increased $88 million in 2019 to $457 million as of December 31, 2019 from $369 million as of December 31, 2018. Our uses of cash totaled $124 million and included cash operating expenses of $118 million and capital expenditures of $6 million. Over the course of the year, we grew the size of our organization by approximately 48%, increasing to 195 full-time employees from 132 at the end of 2018.

The growth in our spending in 2019 with the expansion and maturation of the pipeline and advancement of our platform. We expect these to continue to be the primary driver of spending growth in 2020. Our sources of cash in 2019 totaled $212 million and consisted primarily of $116 million raised through equity issuance, $76 million of milestone payments from our business development partners and $15 million in proceeds from stock option exercises.

Editas is in a strong financial position with at least 24 months of run way to fund the business.

And with that, I will hand it back to Cindy.

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

Thank you, Michelle. We are confident in our strong leadership to guide the company as we become a clinical-stage biotech and embark on our next phase of growth. It has been a busy past few months for Editas and an exciting time as we look towards the coming year.

We have filled out our executive team and are confident the strengthened leadership

Will support the long-term growth of the organization. Our best-in-class programs, coupled with the unparalleled discovery research from our labs, represent a pipeline of transformational medicines for diseases of unmet need. We are eager to see what 2020 and beyond will hold as we look to deliver on the promise of CRISPR to transform patients lives. We thank all of you for your interest and support.

With that, we will open up the call for Q&A. Operator?

QUESTIONS AND ANSWERS

Operator

(Operator Instructions) And our first question comes from the line of Steve Seedhouse with Raymond James.

Steven James Seedhouse - *Raymond James & Associates, Inc., Research Division - Research Analyst*

My question is, at ASH -- so the data that was presented for EDIT-301, it's a nice data set and you included sort of like the iterations on optimizing the protocol, whether it was the enzyme variant or the guide or the complexation conditions and electroporation conditions as well. So there's -- basically, there's a lot of moving pieces there. And I wanted to understand, if we set aside the fact that you guys are targeting a different locus and some of the first movers in the gene editing approaches. I'm curious if you could characterize to what extent you believe the other optimized conditions, so like the electroporation and RNP complexation are novel or are an improvement over what's already been done? Or does the best-in-class pitch for EDIT-301 just boil down basically to the different locus that you're targeting?

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Steve, this is Charlie. The best-in-class comes from a couple of things. One, we believe we have superior levels of fetal hemoglobin induction. Secondly, we believe we don't carry in the potential baggage that comes with adding the BCL11A enhancer.

As you appreciate, you can't knock out BCL11A because that causes dire consequences. And we in our preclinical models have found issues with even the BCL11A enhancer. So we've gone at it after a site in front of the hemoglobin locus, we know is genetically validated. And we scan the entire hemoglobin locus, and we found sites that we thought could get the most fetal hemoglobin induction, then we look for the enzyme combinations would do that.

So as you appreciate, they are both productive and nonproductive edits any time you cut. And we found that cutting at the site we did with Cas12a yielded a lot more productive edits than being cutting with Cas9. But to make that work as a product, we needed to optimize cutting with Cas12a, and we did that, as you mentioned, with a variety of methods. We have a enzyme as a variant of Cas12a that has increased activity, we have variations of the guide that increase the ability to do the editing, and we've optimized the electroporation conditions.

And so all of those things went into building the product.

Steven James Seedhouse - *Raymond James & Associates, Inc., Research Division - Research Analyst*

Okay. That helps just to understand the sequence of events and the thought process quite a bit. I want to also ask on EDIT-301. If the studies that remain ongoing are the gating factors to an IND comprise any primate studies? And is it -- if not, is it worth testing the beta-globin targeting approach in primates, or are rodent model sufficient to sort of see differences in biology between that versus the first generation approaches?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

We don't have any ongoing primate study. So to do the primate study, you actually have to redesign the entire product because it has to be able to cut the primate genome. The -- it's not clear yet whether the primate studies are going to be more predictive than the rodent studies or not. And so at this point, we feel like the thing to do is to get into the clinic as quickly as possible because it's going to be the clinical data that actually determines the course with all these class of medicines.

Steven James Seedhouse - *Raymond James & Associates, Inc., Research Division - Research Analyst*

Okay. And just last quick one. Just -- looks like operating expenses went up considerably in the quarter. Was just curious if you could shed some light on how that breaks out and what would carry forward into next year on a go-forward basis?

Michelle Robertson - *Editas Medicine, Inc. - CFO, Principal Accounting Officer, Treasurer & Assistant Secretary*

Sure. As the programs advance, we'll continue to invest, especially as they go into the clinic. And we expect that to continue in 2020.

Operator

And our next question comes from the line of Amanda Murphy with BTIG.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Amanda Louise Murphy - *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

I just had a few more questions around the oncology business. And I wanted to start out with the, I guess, now BMS relationship. You kind of specifically talked about allogeneic cells, and I'm not sure what you can share there in terms of how that's progressing. But just curious, overall, there's obviously a lot of competition there. You've been working on that for a number of years, they clearly re-upped with you and are interested in the program. So just wondering what your thoughts there on either there's a ton of data coming this year? How those programs might progress? And I want to shift after that on to all the sort of in-house programs that you're working on, but let's start with alpha-beta as we can to start with.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Okay. Amanda, it's Charlie. The -- yes, we did re-up with BMS. We -- they are the leaders in cell-based medicines for hematologic indications. And I think there's a good chance that their CD19 and BCMA programs are going to be industry-leading. We can't share the details of what's going on there. We can say in a more generic way that -- I think that from a light perspective, they're going to want to back up those programs and maintain their leadership. And so we've disclosed a lot of data through the years about the types of edits we've been able to make in T cells. And I think it's safe to assume they're going to want to use some of those same targets, but we're not at liberty to disclose what the detailed products are.

Amanda Louise Murphy - *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

Yes. Fair enough. And then, I mean, then obviously you've been building out quite a -- well sizable portfolio when you think about what you've done with NK cells and the Sandhill now with expansion in a -- and my understanding is that's kind of 1 of the key challenges of using NK cell is expansion technology. And so you talked about, obviously, working with Sandhill and BlueRock spend out, and then I think as part of the BMS revised agreement, you got back gamma delta cells. So would love to just kind of get a high-level view of how you're thinking about the sort of innate effector cells as backbones. And you talked about solid tumor as a target, but just taking a step back from a high level pipeline perspective, how do you see this evolving over time? And what else do you think is interesting? I think Tregs have also been discussed by some other companies, et cetera. So love to get perspective there.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Yes. We'll take a stab at that. So we like the innate immune system. And so we feel like NK cells are the place to start, and I'll come back to gamma delta in a minute. So we know that the NK cells are part of antibody-directed cellular cytotoxicity or ADCC, which is part of the mechanism of your therapeutic antibodies, things such as Herceptin and Erbitux are some among others.

And we also know that NK cells have a very low propensity for graft versus host. So when it comes to making truly allogeneic medicines, they come already in a good place. And we further know that the path to making iPSC-derived NK is reasonably well worked out at this point. So all those things we've taken together and are leading us to go after the major unmet need, which is solid tumors. We think that the NK cells there are exhausted as the T cells are exhausted in any of the solid tumor indications as well. And there are things that we can do about that via gene editing.

And so among the things we want to do is increase sensitivity to IL-15, targeting like the CD16 pathway better overcome the tumor microenvironment and make them truly allogeneic. So all those things are possible with gene editing. The number of those you can do is limited with healthy donor and there in lies the advantage of the iPSC platform, not only do they ease of making the cells, ultimately become much better, but your ability to construct highly engineered medicines, which we think are going to be needed to be successful in this space are enabled. So we are in the process of industrializing the platform, which is a combination of the editing platform we built over the last 5 years with an iPSC platform that we've got to jump-start with by getting cell lines from BlueRock Therapeutics.

Did that get you where you want to go?

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Amanda Louise Murphy - *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

Yes. I think I asked like way too questions in there. But I think also just talking about expansion, that seems to be something that is a challenge with using NK cells. So I wanted to take a little more perspective there and then also gamma delta.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Yes. So that's part of what we get for it -- it's more of an issue for healthy donor than it is for iPSC-derived cells where you can just grow a whole lot. And so that's part of what Sandhill brings to the plate for us. So -- and that's going to help enable our healthy donor program, which will begin to let us learn about what these different edits do in the context of solid tumors.

Amanda Louise Murphy - *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

So it's fair to say that -- yes, sorry, go ahead, I'm sorry.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Gamma delta is another part of the innate immune system. Obviously, it's less the biology of gamma delta cells is less well worked out than NK cells are. But they remain an interesting area. And among the reasons they're interesting is because the relationship between the number of gamma delta cells you see in your solid tumor and the control of that tumor are among the best things that are correlated in the control of solid tumor growth. So it's an interesting area. We're glad to have the ability to now work in that area, and you'll hear more about that in the coming months.

Amanda Louise Murphy - *BTIG, LLC, Research Division - MD & Senior Biotechnology Equity Analyst*

Is it fair to say you're using different approaches to really tackle solid tumor using edited engineered effector cells? So that -- is that the right way to think about it as a strategic (inaudible)for you?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Yes. Yes. Yes. And we're trying to -- and I would further say, we're trying to take the natural properties of NK cells and make them and restore them and make them better. And so NK cells, as I told you, participated in antibody-directed cellular cytotoxicity or ADCC. We also recognize cells that are lacking MHC. So thereby, don't express T cell antigens. And so 1 of the major resistance mechanisms to PD-1 inhibitors is the loss of T cell antigen expression. So if you had a therapeutic that could specifically target those cells, you have a nice add-on to PD-1.

Operator

And our next question comes from the line of Matthew Harrison with Morgan Stanley.

Thomas Francis Lavery - *Morgan Stanley, Research Division - Research Associate*

This is Thomas Lavery from Matthew's team. I have a question about EDIT-102. When does your partner, Allergan, need to decide on next steps? And if they don't opt in, what would be your plans to pursue a trial alone?

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Sure. So the way the deal is structured is that we agree on the targets and the option criteria with Allergan before we start a program. And then we go away, we develop the medicine that fulfills the criteria and then we deliver that option package, essentially at what would be known as a developing candidate stage. So it's ready for IND-enabling tox study. And then Allergan has a period of time to evaluate that package and make a decision about whether they opt-in or not opt in. And then after that, we have a window to decide whether we co-opt in with them to co-develop in the product with them. We did that for LCA10. And if they decide not to opt in, the medicine comes back to us, then we have the right to develop it on our own.

Operator

Our next question comes from the line of Gena Wang with Barclays.

Unidentified Analyst

This is [June] for Gena. So my first question is really about the LCA10. Could you give us, first of all, some guidance about when the clinical data will be released? That's first.

And then also, can you give us a little bit of color about the range of your initial dose you would have to pay? And also, what are kind of biomarkers you would be looking for that will help you to decide whether you're going to move on to the higher dose? I will have a follow-up after that.

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

Okay. Thank you for the question. So I'll start with the first part of it and ask Charlie to talk a little bit more about biomarkers and dose range. But the overall trial is going very well. We are actively screening patients and have not disclosed when we will announce data at this point in time. As I mentioned earlier, we expect to announce dosing of the first patient this quarter, but we have not made any commitment around when we might think about sharing data. Charlie, do you want to...?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Sure. And so we picked doses based on our preclinical studies. They -- the low dose, we have a low a mid and a high dose, they are -- they span the dose that's used by Spark in the LUXTURNA trials, it was found to be safe and efficacious. The -- and it's a relatively narrow dose range. The lowest dose has a realistic chance of showing efficacy based on the preclinical studies, and you have to do that for a gene therapy trial. The -- we're looking at a range of clinical outcomes. Some of them are clinical, their visual acuity, as well as electrophysiologic and structural markers, including ERG and OCT, and we'll take all that data into consideration as well as the safety and as we decide how to advance the program.

Unidentified Analyst

Great. Just to follow-on that, how long are you generally going to wait after dose your -- your lowest dosing of patients before you think that you have enough information to decide whether to move on to the higher dose? What's the time, waiting time between the 2 doses?

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

So we have a safety review at the conclusion of cohort 1. And that review period can be -- program to be approximately 6 weeks in duration.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Unidentified Analyst

Okay. So I have another question about the sickle cell disease. Obviously, you have your preclinical data from ASH, and you also -- you elaborated about the advantages you have. So just for kind of giving myself a little benchmark of understanding, what level of editing efficacy you think will be something give you confident your program would be better for like best-in-class, as you suggested? What the percentage of gene editing you are looking for to move forward?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

I think it's not so much of gene editing, it's just fetal hemoglobin induction. So we're obviously going to optimize the percent editing based on the technical features. But really, what we're looking for are levels of fetal hemoglobin and then that would be predictive of clinical outcomes.

Operator

And our next question comes from the line of Joe Thome with Cowen & Company.

Joseph John-Charles Thome - *Cowen and Company, LLC, Research Division - VP of Healthcare*

On the EDIT-101 study, can you just let us know if -- has the first patient surgery been scheduled, and maybe what needs to happen before the patient is dosed? And then looking forward, kind of towards the end of the year, do you still expect the yield of dose kind of the first 2 cohorts of patients in that study by year-end?

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

Yes. Thank you for the question. So as I mentioned, the trial is going very well. We are actively screening patients. As you've heard from us previously, the screening and enrollment has been a little bit more complicated than other types of trials, just getting patients lined up with family members, caregivers, things like that. But we are in daily contact with our partner, Allergan, and working very collaboratively to continue to screen the patients. So I feel very good about where we are. We had said earlier that we do hope to treat both the first 2 cohorts by year-end, and we're still tracking towards that.

Joseph John-Charles Thome - *Cowen and Company, LLC, Research Division - VP of Healthcare*

Okay, great. And then 1 more kind of just on a more broad strategy. Obviously, you have a lot of partnered and collaborative programs. When you're looking at going into a new therapeutic area, I guess, how important is it to have a partner that you can kind of lend on their expertise and combine your strengths versus deciding to do a program alone?

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

We've taken those, obviously, each 1 in isolation. And so we have partners such as Allergan, where we have the extreme -- where we are codeveloping and co-commercializing potential therapies. The partnership now with BMS, formerly Celgene-Juno was a little bit different in that it was a development collaboration, but our role is unique there. We weren't co-coing products, per say. With some of the more recent deals that we've done, Sandhill and BlueRock, and to some degree, AskBio, those were really purposeful in terms of getting access to either technology or capabilities that we thought were important to facilitate getting the programs up and running and going much more quickly.

Operator

And our next question comes from the line of Geulah Livshits with Chardan.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Geulah Livshits - *Chardan Capital Markets, LLC, Research Division - Senior Research Analyst*

So to follow-up on that, on to the prior question on EDIT-101. So I think at the third quarter results, it was mentioned that a patient has been identified. The (inaudible) stick to additionally some of the factors around the timing of dosing and is that the same patient the 1 that's still expected to be dosed? And then I have a couple of follow-ups.

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

So I'm not sure I recall specifically the statement about first patient being identified, per say. But we are, as I've said, actively screening patients. We have identified patients for the first cohort and are working even towards the other cohorts. So best I can say at this point is that we do expect to announce dosing of first patient this quarter.

Geulah Livshits - *Chardan Capital Markets, LLC, Research Division - Senior Research Analyst*

Great. And then actually, to follow-up on some of the questions regarding the oncology programs. Can you elaborate a little bit more on the Sandhill collaboration? And what drew you to work with that company? Then I have a follow-up on that.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Sure. Sandhill has developed a technology they call BINATE, which allows the efficient expansion of NK cells, which is -- was -- as referred to earlier, a significant issue in that field. So that's the primary driver of that. It's a group that has experience in developing cell-based medicines as part of earlier companies, and we felt like they could be a good partner in the NK field because of the technology they develop there.

Geulah Livshits - *Chardan Capital Markets, LLC, Research Division - Senior Research Analyst*

And is that tech also applicable to the gamma delta cells? Or is that primarily for the NK program?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

There are elements that may be applicable to gamma delta, but the primary use in the short run is for NK cells.

Geulah Livshits - *Chardan Capital Markets, LLC, Research Division - Senior Research Analyst*

And then I think at some point, you previously had a collaboration with Gamida Cell, I think, around NK technology. So can you remind us, is that something that's still active? Or is this the Sandhill collaborations to percieve that? Or do both the approaches come into play for a donor-derived program?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Yes. Those were independent collaborations, and we had an MTA with Gamida Cell.

Operator

And our next question comes from the line of Silvan Tuerkcan with Oppenheimer.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Silvan Can Tuerkcan - *Oppenheimer & Co. Inc., Research Division - Associate*

Congrats on the quarter. My first question is, again, about the LCA10 data, when we eventually get it? Probably towards the end of the year? How comparable or what can we compare across to the Phase I/II clinical trial for -- by ProQR, just in terms of -- is it similar patients, is it not? What's comparable, what's not? If you could give us some color there.

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

So to comment on data, I do anticipate that there is the potential to have some data to share by the end of the year that's been our previous guidance. I'll ask Charlie to comment on the ProQR portion of the question.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

The patients are relatively comparable. That's the short answer to your question, there's not huge differences. I wouldn't know if there are detailed differences, but they're in the same ballpark.

Silvan Can Tuerkcan - *Oppenheimer & Co. Inc., Research Division - Associate*

And maybe 1 question for Michelle. As you're taking on the role of the CFO, could you maybe help us understand how you view the different programs in eye, oncology and D&S now and sickle cell in terms of maybe on the dimension of risk versus payoff versus how you would allocate assets towards them?

Michelle Robertson - *Editas Medicine, Inc. - CFO, Principal Accounting Officer, Treasurer & Assistant Secretary*

Sure. I feel I'm getting up the speed on the portfolio. But I think that we're comfortable with the 2 pillars that we've talked about publicly at JP Morgan and of the programs and the relationships that we have with our partners. I think that what we'll continue to do is assess our portfolio as a whole, so that we can prioritize the programs and our investments and map out our timelines in the events. And as we think about financing and supporting the portfolio, I believe that prioritization is going to be sort of a key for the next 12 to 24 months because we have such a good pipeline, and that we're going to have to, I think, focus in on the high-value programs, working closely with our partners.

Silvan Can Tuerkcan - *Oppenheimer & Co. Inc., Research Division - Associate*

Great. And maybe 1 last question here on this AskBio, CNS partnership. What are kind of the indications that you're going after in the long term?

Michelle Robertson - *Editas Medicine, Inc. - CFO, Principal Accounting Officer, Treasurer & Assistant Secretary*

We have not yet disclosed the indication that we're pursuing with them.

Operator

(Operator Instructions) And our next question comes from the line of Yanan Zhu with Wells Fargo Securities.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

Yanan Zhu - *Wells Fargo Securities, LLC, Research Division - Associate Analyst*

So first question is on EDIT-301. Would you, at some point, share a preclinical data comparing your gamma-globin promoter targeted approach versus the BCL11A enhancer approach? And also, just hypothetically speaking, is there anything could be gained if the 2 targeting approaches are combined? Or do you think the gamma-globin promoter approach achieves maximum HBF induction already?

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

Sure. Answer to your first question is yes, at some point, we'll disclose the comparative data, but I'm not sure when that will be.

Combining them is an interesting question. The -- I think there is some technical challenges of doing that, I guess, that wouldn't be our first move is, I guess, you'd have to edit -- you have to do both editing events at the same time, there'll be some technical challenges that come with that.

Yanan Zhu - *Wells Fargo Securities, LLC, Research Division - Associate Analyst*

Right. Got it. Just -- yes. So the purpose of that question is mainly to see whether the 2 approach has -- there are HBF inductions that could be achieved by independently different mechanisms? Or perhaps they are working in the same pathway, and 1 is better than the other. But yes, I agree that it's not a real proposal, but rather to understand the pathways. But maybe a quick question on the NK cell program. In terms of the kinds of edits you're doing, I think you mentioned you're editing to increase potency of NK cell killing. Would edits -- any edits to increase persistence, also something that you're considering? Or do you see this mainly as a multiple dosing approach and therefore persistence? It doesn't really come into the equation.

Charles Albright - *Editas Medicine, Inc. - Executive VP & Chief Scientific Officer*

We will make edits to increase persistence even and multiple dosing still remains on the table as well.

Operator

And this concludes today's question-and-answer session. I would now like to turn the call back to CEO, Cindy Collins, for closing remarks.

Cynthia L. Collins - *Editas Medicine, Inc. - President, CEO & Director*

Great. So with that, we thank you for participating in today's call and for your support as we work to bring transformative new medicines to patients. Have a great evening.

Operator

Ladies and gentlemen, this concludes today's conference call. Thank you for participating. You may now disconnect.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS

DISCLAIMER

Thomson Reuters reserves the right to make changes to documents, content, or other information on this web site without obligation to notify any person of such changes.

In the conference calls upon which Event Transcripts are based, companies may make projections or other forward-looking statements regarding a variety of items. Such forward-looking statements are based upon current expectations and involve risks and uncertainties. Actual results may differ materially from those stated in any forward-looking statement based on a number of important factors and risks, which are more specifically identified in the companies' most recent SEC filings. Although the companies may indicate and believe that the assumptions underlying the forward-looking statements are reasonable, any of the assumptions could prove inaccurate or incorrect and, therefore, there can be no assurance that the results contemplated in the forward-looking statements will be realized.

THE INFORMATION CONTAINED IN EVENT TRANSCRIPTS IS A TEXTUAL REPRESENTATION OF THE APPLICABLE COMPANY'S CONFERENCE CALL AND WHILE EFFORTS ARE MADE TO PROVIDE AN ACCURATE TRANSCRIPTION, THERE MAY BE MATERIAL ERRORS, OMISSIONS, OR INACCURACIES IN THE REPORTING OF THE SUBSTANCE OF THE CONFERENCE CALLS. IN NO WAY DOES THOMSON REUTERS OR THE APPLICABLE COMPANY ASSUME ANY RESPONSIBILITY FOR ANY INVESTMENT OR OTHER DECISIONS MADE BASED UPON THE INFORMATION PROVIDED ON THIS WEB SITE OR IN ANY EVENT TRANSCRIPT. USERS ARE ADVISED TO REVIEW THE APPLICABLE COMPANY'S CONFERENCE CALL ITSELF AND THE APPLICABLE COMPANY'S SEC FILINGS BEFORE MAKING ANY INVESTMENT OR OTHER DECISIONS.

©2020, Thomson Reuters. All Rights Reserved.

©2020 Thomson Reuters. All rights reserved. Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks of Thomson Reuters and its affiliated companies.

THOMSON REUTERS