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PRESENTATION

Cory William Kasimov *- JP Morgan Chase & Co, Research Division - Senior Biotechnology Analyst*

All right. Good morning, everyone. My name is Cory Kasimov. I'm a senior biotech analyst at JPMorgan. And it's my pleasure to introduce our next company, Editas. Here to present for Editas is the company's CEO, Cindy Collins. And please note that following Cindy's presentation, there's a breakout across the hall in the Borgia room.

So with that, I turn it over to Cindy.

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

Thank you, Cory, and thanks for the invitation to speak here today. Pushing the boundaries of medicine entails risk as well as great promise. This slide tells you all about the risk. At Editas, we strive to make differentiated, transformational medicines for diseases of high unmet need. To accomplish this objective, we are pursuing 2 strategic pillars, in vivo CRISPR medicine and engineered cell medicines. In our in vivo CRISPR medicine portfolio, we are able to leverage AAV-mediating editing with our proprietary staff Staph aureus Cas9 into several therapeutic areas.

For an engineered cell medicines, we are developing best-in-class medicines for hemoglobinopathies with our proprietary Cas12a and solid tumors using our iPSC-derived cells. Over the past years, we have invested heavily in our platform and can now leverage this effort to advance both in vivo and ex vivo cell medicines. We have a strong IP portfolio and continue to advance our organizational capabilities.

2019 was a very productive year for Editas. For our in vivo CRISPR medicines, we initiated the first-ever clinical trial for EDIT-101, established in vivo proof-of-concept and declared EDIT-102 a development candidate for USH2A. We also advanced our RP4 program, and we expanded into neurological diseases with a partnership with AskBio.

For engineered cell medicines, we initiated IND-enabling activities and presented our preclinical data for EDIT-301 for sickle cell disease and beta thalassemia. In oncology, we refocused our BMS-Celgene collaboration on alpha-beta T cells exclusively, thereby returning gamma delta rights to Editas. We advanced our iPSC-derived cell medicines for solid tumors using technology from BlueRock Therapeutics, and we generated NK cells from healthy donors and iPSCs with significantly increased antitumor activity.

On the organizational front, we hired a Chief Executive Officer, a Chief Medical Officer, a Chief Financial Officer and an SVP of Operations. We grew the employee base to 196 employees. And to support these efforts, we brought in an additional $75 million in cash through business development activities. These efforts plus early work lead to the following portfolio. You can see that our most advanced programs are EDIT-101, EDIT-301 and our healthy donor NK program. The remainder of the portfolio is progressing nicely.

We expect 2020 to be another great year for Editas. For our in vivo CRISPR medicines, we aim to dose the first patient with EDIT-101 this quarter and complete the low and mid doses in adults by year-end. We plan to establish in vivo proof-of-concept for a neurological indication and nominated

development candidate for RP4. For engineered cell medicines, we aim to file an IND for EDIT-301 by the end of the year. For oncology, we will initiate IND-enabling studies for an engineered healthy donor cell medicine to treat solid tumors. We will establish in vivo proof-of-concept for an engineered iPSC-derived NK cell, and we will advance the alpha-beta T cell medicine portfolio with BMS. We are building out our clinical and medical affairs organization and advancing manufacturing and operations to support our clinical activities.

Now turning to our ocular programs. Our lead program aims to benefit patients with LCA10. We are codeveloping & co-commercializing this program with our partner, Allergan. While patients have a wide range of visual acuity, many of these patients are essentially born blind. The visual defect is due to a mutation in the gene that encodes for the CEP290 protein. We expect to dose our first patient this quarter.

The CEP290 protein is essential for photoreceptor function. In particular, photoreceptors lacking the CEP290 protein have defective outer segments. Outer segments are the portion of the photoreceptor that sense light. In you and I, these turnover about every 10 to 14 days. By correcting the splicing defect in the gene encoding for the protein, we expect that these photoreceptors will regrow the outer segment and restore light sensitivity and hopefully vision.

We've presented a lot of preclinical data on EDIT-101, including our publication in nature medicine. I wanted to highlight a couple of pieces of data from this publication. On the left graph, we show that editing by EDIT-101 in mice that contain a portion of the human genome that is sensitive to EDIT-101. This data shows that editing is completed within 6 weeks and was sustained over the 26 weeks of the experiment. We have similar results in nonhuman primates. For these reasons, we expect that the editing will occur within a few weeks in humans and lead to visual changes in a few months. On the right side of the graph, we show the dose response curve is very steep. Since we will begin in doses that could be therapeutically useful, the steep dose relationship limits the number of doses that we -- that will have to be administered, thereby accelerating the program. The Phase I/II interventional study, termed Brilliance, is typical for a rare disease. The study is an open-label, dose-escalation study where we look at safety, tolerability and efficacy. We will use surgical sites that were used in the Spark trial to reduce the procedural risk. The trial is currently enrolling. We will start in adult patients with light perception only and at a dose that is likely at the lower end of the therapeutic range. Based on safety and tolerability in these patients, we will escalate and eventually move into children with some visual acuity. We have a variety of clinical endpoints built into this study. We will look at the data in real-time with our partner, Allergan, and we aim to dose both the low- and mid-dose cohorts in adults this year with the possibility of data disclosure this year as well.

Our second ocular program is aimed at USH2A, which, like LCA10, is an inherited retinal disease affecting the photoreceptors. As a result, our USH2A program benefits tremendously from the work that was done on EDIT-101. In particular, we plan to use the same vector, the same Cas9 and the same promoter. We believe this de-risk and accelerates the path to the clinic and ultimately, a transformative medicine for patients. We just cleared a development candidate for EDIT-102 at the end of last year.

This slide summarizes the editing strategy and data supporting the biology for the approach. Since most of the mutations for USH2A are found in exon 13, we tested an exon 13 deletion strategy for therapeutic benefit. In the middle panel, we show that the editing removes exon 13 and generates the new messenger RNA in human retinal explants, as expected. On the right panel, we show that the editing restores USH2A complex and morphology to human organoids. Taken together, these data support our editing approach.

Our third ocular program is aimed at RP4. RP4 is a progressive cause of blindness due to the accumulation of defective rhodopsin protein in photoreceptors. RP4 is a relatively common form of inherited retinal disease affecting approximately 26,000 patients. Since RP4 mutations are found throughout the gene, we will knock out the endogenous protein and replace it with a wild-type version. We have made good progress on this program in 2019 and are aiming for development candidate in 2020.

As you can see from the progression of our ocular programs, we leverage learnings for subsequent programs, thereby allowing us to create clinical molecules with increased efficacy. We now plan to leverage our ocular learnings into new tissues that are accessible by AAV. We have exclusive and proprietary access to Staph aureus Cas9, which can be packaged into a single AAV with 2 guides and required regulatory elements. As we stated earlier, we started a collaboration with AskBio for a neurological disease. There are multiple other neurological diseases that could be approached based on our experience in this area. Likewise, we outlined a progression of other tissues where editing can be beneficial. For these reasons, we view our in vivo CRISPR medicine as 1 of our 2 pillars of our therapeutic strategy.

Now I'd like to transition to our engineered cell medicines, the second pillar of our therapeutic strategy. Let's start with our editing of hemo-poetic stem cells for beta hemoglobinopathies, including sickle cell disease and beta thalassemia. These diseases are areas of high unmet need with a clear understanding of the underlying biology. In particular, we know from human biology that elevated fetal hemoglobin can dramatically reduce the symptoms for these diseases. We have identified a development candidate that robustly induced fetal hemoglobin and have begun IND-enabling activities. We are on track to file an IND by the end of this year.

This slide summarizes why we believe that EDIT-301 can be best-in-class. Starting at the top, we believe that EDIT-301 has the potential to impact beta globin in the best way. In particular, the preclinical data shows that we induced more fetal hemoglobin than the BCL11A enhancer approach. And relative to lentiviral gene therapy, gene editing will reduce the sickle cell globin and, therefore, not have to compete with alpha globin in the same cell.

On the safety side, editing at the globin locus but not at the BCL11A locus is supported by human genetics. Our preclinical studies identify one potential concern for BCL11A editing as we found deleterious lineage skewing when editing the BCL11A locus.

Finally, gene editing is more specific than lentiviral expression to get the high levels of beta globin required for efficacy. These cells in the CD34 population will likely carry up to 20 copies of the viral genome. These random integration events have the potential to inadvertently activate or inactivate genes involved in tumorigenesis. Based on our belief that editing at the beta-globin locus was the preferred therapeutic approach, we conducted a comprehensive screen of the beta-globin locus for sites that would elevate fetal hemoglobin. In particular, we screened over 26,000 guides over a 300 kilobase region of the beta-globin locus. This screen was successful in identifying several sites, including those predicted by human genetics that elevate fetal hemoglobin. We then interrogated whether Cas9 or Cas12A was the preferred editing enzyme. On the left panel, we show that indels greater than 3 nucleotides will induce fetal hemoglobin more than small deletions. In the middle panel, we show that indels created by NHEJ repair process are preferentially retained compared to indels created by MMEJ repair process. This finding suggests that MMEJ is not very active in the fraction of the CD34 cells that are long-term stem cells. On the right, we show that Cas12A induces more NHEJ indels than Cas9.

We then compared editing at the beta-globin locus to editing at the BCL11A locus. As expected, editing at the beta-globin locus was equivalent to unedited cells in terms of repopulating the erythroid lineage as shown on the left side of the slide. In contrast, cells edited at the BCL11 enhancer had a significant reduction in cells of the erythroid lineage. If this reduction was observed in humans, then the efficacy of the BCL11A enhancer approach would be compromised. To further investigate the mechanism of differences between editing at these 2 sites, we measured the levels of apoptotic cells and found that the BCL11A enhancer edited cells had an increased level of cell death.

We also tested the ability of cells edited at the beta-globin locus to induce fetal hemoglobin. As we predicted from our in vitro studies, editing at the beta globin site with Cas12 caused a robust induction of fetal hemoglobin with approximately 45% above background levels. Encouragingly, fetal hemoglobin induction was pan cellular, as shown on the right side. Taken together, we believe that EDIT-301 has the potential to be a best-in-class medicine, and we are excited to progress it to an IND this year.

I'd like to now switch gears to our oncology efforts, a significant and growing portion of our portfolio. This slide shows some of our thinking about the evolution of cell-based therapies. Starting on the left side are the autologous therapies, where the approved medicines for oncology fit today. While effective, these medicines are complex and expensive to manufacture and create tremendous logistical challenges. Further, the ability to edit these cells to improve their properties is limited. The creation of allogeneic medicines as depicted in the middle panel from healthy donor pools would be a significant advance in that these therapies would be off the shelf. However, the amount of genomic changes will still be limited and technical challenges involving batch-to-batch standardization remain. On the right side of the slide are medicines derived from iPSCs. These medicines provide off-the-shelf convenience with the ability to perform essentially infinite number of genetic changes.

With this vision for the cell therapy space, there are several cell types that are potentially useful for oncology. Alpha-beta T cells are part of our adaptive immune system and have proven therapeutic for liquid tumors. These cells recognize tumors either from the endogenous alpha-beta T cell receptors or synthetic chimeric antigen receptors, or CARs. While we can edit these cells to construct allogeneic medicines, the potential for graft-versus-host disease from contaminating cells remains an issue. Furthermore, the number of edits required to make these cells allogeneic will consume most of the potential for genetic changes in these cells. For these reasons, we are focusing our efforts for our wholly-owned programs

on innate immune cells, particularly gamma delta T cells and NK cells. While we'll have more to say about gamma delta T cells in the future, I want to focus today on NK cells.

NK cells can recognize tumors by a variety of mechanisms, including multiple innate receptors that recognize cells that don't express T cell antigens in cells that express stress ligands. NK cells are also an important part of the mechanism by which many therapeutic antibodies kill tumor cells in a process known as antibody-directed cellular cytotoxicity, or ADCC. Further, NK cells can be engineered with CARs. And importantly, NK cells do not cause graft-versus-host disease. Among the appealing features of NK cells is that ADCC and innate receptors are part of what allows NK cells to participate in the killing of solid tumors and, therefore, are our desired cell type since our efforts will focus on the high unmet need in solid tumors. As part of our first set of products, we will improve the ability of NK cells to kill by ADCC. In particular, we will increase the signaling power of the ADCC pathway, improve the persistence of NK cells and edit cells to overcome the suppressive nature of the tumor microenvironment. These products will pair with therapeutic antibodies, such as anti-HER2 and anti-EGFR. In the next set of products, we will improve the ability of NK cells to kill tumors via innate receptors and CARs. NK cells that kill tumors lacking T cell antigens will be well suited for pairing with anti-PD-1 therapies since a major mechanism of PD-1 nonresponse is due to the lack of T cell expression.

This slide shows some of our initial work on NK cells from healthy donors. As shown on the left, we can efficiently edit these cells with single and double knockout efficacies exceeding 80%. These edited cells are more potent in killing tumor cells in vitro as shown on the right. In vivo studies are ongoing and are expected to show a similar improvement in potency. We are on track to declare a development candidate for this medicine and initiate IND-enabling activities this year.

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While we feel that edited healthy donor-derived NK cells will be a significant advancement in the field, we believe that iPSC cells are required to unlock the full potential of cell medicines for oncology. This slide depicts the process of making iPS-derived cells and some of the progress we've made in the last few months. Starting on the left, iPSCs are created from somatic cells by de-differentiation. These cells must then be edited and characterized so that a clonal cell line can be selected. We have optimized editing conditions with our proprietary Cas12A derivative. Further, we have built in an unparalleled ability to characterize the genome of edited cells. We will then take these edited clonal lines and differentiate them into iPSC-derived NK cells using a proprietary and scalable process. The end result will be cells that are more highly engineered than is possible than from other cell sources with a low cost of goods and available off-the-shelf in perpetuity.

On this slide, we show some of our early results with iPSC-derived NK cells. The left graph shows that we can efficiently edit a variety of genomic targets. The right graph shows that edited cells are more potent at killing tumors than unedited cells.

Partnerships have been and will continue to be an important part of building our leadership position. We have 2 important development and commercialization partnerships. The first is with Allergan, our partner in ophthalmology. They've been a tremendous partner. They have deep expertise in ophthalmology, and we've benefited from that. Recently, we amended our partnership with Celgene, now BMS, to just focus on alpha-beta T cells giving back the gamma delta rights to Editas. And then in 2019, we signed 2 new important collaborations. We signed a deal with AskBio in neurological diseases, and we signed a deal with BlueRock Therapeutics to gain access to technology and GMP, iPSC cell lines. And then just this week, we announced a deal with Sandhill Therapeutics to access their proprietary binate technology to develop healthy donor NK cells for solid tumors. We also underpan our business with a very strong intellectual property position. We invest in this because we think it's important to the field long term.

To close, we are making progress on both pillars of our therapeutic strategy to create differentiated transformational medicines for diseases for high unmet need using our proprietary Staph aureus Cas9 and Cas12A. For our in vivo medicines, we are advancing EDIT-101, the first-ever in vivo CRISPR medicine into the clinic. In addition, we are progressing our ocular program and moving into other therapeutic areas such as neurological diseases. For our engineered cell medicines, our lead program is EDIT-301 for sickle cell disease and beta thalassemia. We continue to believe that EDIT-301 is potentially best-in-class. We also have significant efforts aimed at solid tumors with both healthy donor and iPS-derived NK cells.

So thank you for your attention, and we'll be happy to entertain questions across the hall.

QUESTIONS AND ANSWERS

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

Okay. So good afternoon, everyone. Again, I'm Cindy Collins, the CEO of Editas Medicine. To my right is Judith Abrams, our new Chief Medical Officer; to my left, Charlie Albright, our Chief Scientific Officer; and to his left, Michelle Robertson, who is our new Chief Financial Officer.

Unidentified Analyst

So I will start with all the new officers, I guess. First question is how disruptive, if at all, are all the changes at the highest level? (inaudible) all the work that's been done?

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

So from my perspective, not disruptive at all. It was a highly unusual situation, of course, where we had an interim CEO, myself, and an interim CFO, all the way up until last week. But as you saw from the slides and the information that I presented, we made tremendous progress on all of our goals for the year. And internally, I think we haven't really missed a beat at all. We've been able to hire an enormous number of individuals, almost 70 people in 2019. And then once I came on board as the permanent CEO, we were able to start filling some of the other C-suite slots. But honestly, business as usual internally.

Unidentified Analyst

And then from a program standpoint, maybe starting with EDIT-101, as you (inaudible) and dose your first patient, can you talk about the case of the trial? How quickly this could move your strategy with disseminating data at (inaudible)? And what businesses depend on what you see something material enough to disclose it? Or is there a set time you plan to (inaudible)?

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

So I'll ask Charlie to address the question. But no, there's not a set time, and it will depend on the data for sure.

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

Yes. The trial is a typical gene therapy trial. There'll be intervals between the patients being dosed. As you saw, our aim is to dose the low- and the mid-dose cohorts this year. There's certainly the potential to see data that we would disclose this year. We're not promising that because it depends on what the data is. And recall that the low dose is believed to be therapeutic, but it's the low end of where we expect to see efficacy.

Unidentified Analyst

Cindy, that was a great presentation. The last couple of days, we've heard a number of companies all focusing on sickle cell disease. So from -- as you look at the competitive landscape, is it a field which winner takes it all? Or is it going to be that different companies can be successful in different kinds of treatments of sickle cells?

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

That's a great question. So obviously, we're lagging in terms of being in the clinic. But we do think we have the best-in-class approach, as I outlined in the presentation. And from our perspective, we believe that the best medicine will win the day. And that the adoption even early on with other therapies that might be before us will be slow, but I think patients will pick the best medicine.

Unidentified Analyst

Just to follow-up on that, what does best-in-class mean to you in terms of clinical outcomes in sickle cell in the context of some of the data we've already seen in other programs?

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

So I think that the therapy has to be safe, first and foremost. And then to be able to make an impact on survival -- overall survival, to decrease the vaso-occlusive crisis, a painful crises, and to start to approximate measurements of organ function and reversal or stabilization of deterioration of organ function are all important endpoints along with fairly increasing people-building levels.

Unidentified Analyst

So I guess to follow-up on that. We've seen the data -- (inaudible) data from CRISPR (inaudible) was obviously very immature at this point. But what was seen with Bloomberg and (inaudible) 99% reduction they talked about, is there an expectation because (inaudible) associated with that, that changes (inaudible) difficulty in making better outcome there?

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

So I'll start with the answer. I think that the compelling factor that they're happy to do a Phase III program suggests that there are issues that need to be monitored beyond the data presented. And I believe that, that encompasses the durability of the response because there's some data that was presented at ASH about patients that were required to go back on transfusions after some period of time post the gene therapy. And there's also some safety issues that maybe I'll let Charlie address.

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

We have theoretical issues that we outlined with respect to the -- there's going to be some fraction of the cells that have really high viral copy number, and that's going to be at least a theoretical concern, and we'll see how that plays out from a practical standpoint. And so I think those are all significant issues on top of some of the manufacturing issues, reproducibility issues, the things that go with any gene therapy, particularly lentiviral gene therapy. We've seen some of the variability that happens in the CAR T area. It would be interesting to see what the variability really is patient to patient in the HSC field.

Unidentified Analyst

Just -- I understand the topic and the topic variability. For hemoglobin F-inducing programs, how do you think about the fact that your gene editing is happening versus the fact that you get from just doing a transplant? It's a well-known documented effect that you do get some hemoglobin F induction. And how does that translate to thinking about how long you're going to have to follow these patients within a clinical trial?

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

How long to tell whether your therapy data versus, in essence, the transplant is that part? Absolutely. I think that we don't know for sure, but it's many months. And so I think calling results after 6 months is very risky. 9, 12 months, maybe even longer to really understand what the difference between the initial treatment and transplant versus what the gene therapy actually did. So part of the confusion right now, for instance, if you look at the Sangamo data, there isn't a strong correlation between fetal hemoglobin induction and editing. So it would be nice to see the editing data for all of the CRISPR-based drugs that are out there right now or all the editing drugs that are out there right now. Some of that is not in the public domain right now.

Unidentified Analyst

Can you talk about the development plan for 301 once (inaudible)?

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

We have a question for you.

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

Yes. We're just in dialogue with the regulatory authorities now. But further to what Charlie was mentioning before around endpoints, there is a clear regulatory path that's been set with some of the monoclonal antibodies and small molecules that have been recently approved in the last quarter of last year. And they have proximate endpoints at 1 year, where clinical meaningfulness can be demonstrated based upon painful crises and other endpoints. And so clearly, we will be following and embrace and endorse pathway and that's to be finalized over the next month or so.

Unidentified Analyst

So my question is related with Charlie (inaudible) iPSC, you were using universal allogeneic iPSC for NK cells. Is there a scientific reason that universal iPSC -- universal allogeneic cells are more contagious than (inaudible) cells? And if so, what is that for a disadvantage? And (inaudible) using universal allogeneic cells, is it cost (inaudible) science or what you understand science (inaudible).

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

Sure. There are multiple reasons we like the iPSC platform. And you mentioned some of them in your question. So kind of in reverse order. The cost of goods is going to be a lot lower. And so the estimates from some of our competitors' cost of goods for a dose of an iPSC-derived cell is going to be about $3,000. That's going to be more in line with the typical small molecule drug instead of an autologous cell therapy. But scientifically, one of the things we really like about the iPSCs is the ability to do a lot of editing and end up with a population of cells that we were able to characterize at the genomic level, and we know what they are. And so because we believe that to get into solid tumors, you're going to need to edit multiple things, and we mentioned some of them in the slides, to make cells allogeneic, persist longer, be more potent, evade the tumor microenvironment and several other things. And the tumor microenvironment has multiple suppressors. So you can easily get to well over 5 and probably up to 10 edits of things you probably want to do. And we can disagree about the number of edits that are feasible in an autologous or healthy donor product, but they're probably in the 3 to 4 range. At that point, we'll be getting a lot of translocations, we'll be getting a really mixed cell population, and obviously, we can select against the clones. Those clones that are undesirable, we make the iPSC line -- edited iPSC line from which the product will derive. We've already shown that the edited iPSCs are more potent than the non-edited iPSCs. We didn't show it this way, but the edited iPSCs are now as potent as a healthy donor iPSCs -- edited iPSC. So we believe we can match the potency of the autologous cells with the iPSCs. And that, combined with the advantages of manufacturing, the advantage of being able to make a complex product and iterate around it, where you make the first product with a half a dozen edits, you make the second product with another half a dozen edits on top of that, and the ability to iterate around that, combined with translational medicine, should put us in a fairly unique place.

Unidentified Analyst

Can you talk about how you're working on the iPSC front similar or (inaudible)?

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

Sure. I think that we have a huge advantage because we've built a rigorous editing platform. And so while you can argue that you don't need that much editing, by the time you want to make 5 or 6 edits at the same time, it's nontrivial, but perhaps more importantly, our ability to characterize the genome. We developed a tremendous number of tools look at on and off-target, editing, karyotyping, et cetera. And so to select the right clone out of something where you try to do 5 or 6 edits all at the same time is nontrivial, to follow that clone through time to make sure that it doesn't drift is nontrivial. And so all of the molecular biologic methodologies that we've developed to develop products like EDIT-101 are going to come to bear in the iPSC place -- space. And there's nothing unique about differentiating edited iPSCs and NKs. Those are biologic problems. We've already shown in a lab scale, we can do that with the way that's distinct from what is patented. And so we believe the differentiation part is going to go. We've hired a robust team of oncologists. Hired Rick Morgan a year ago, who led the BCMA program at bluebird, and we've built a really nice team of scientists around him. So we feel well positioned to deliver in this space and are eager to get these things in the clinic.

Unidentified Analyst

To go back to the retinal side of things for a minute, how is there any impact on that part of the business from your partners' strategic distractions that are (inaudible)?

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

Thankfully, no. We haven't noticed any change thus far with the pending acquisition by AbbVie. It's been business as usual. And as I said, they've been a great partner, continue to be a great partner, and we hope that the integration, the acquisition doesn't change that.

Unidentified Analyst

And then can you talk about your next (inaudible). The letter you can get from that (inaudible) remain in the (inaudible).

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

Yes. So as I said in the presentation, we're able to leverage the same promoter, same Cas9 enzyme, same vector. And so that is a tremendous benefit in terms of accelerating the program. You want to speak to the remainder of it?

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

Sure. And on top of those, advantages of the pharmacologic models were analogous. We're approaching the IND-enabling studies in an analogous way. We got the first package through the FDA in 30 days that bless in essence our approach to specificity, toxicology, manufacturing, et cetera. And we were able to do that with a team that was less than half of the size of the LCA10 team. And so that's the typical kind of efficiencies one gets when you get into a therapeutic area, you can build both the technical and the biologic expertise to advance program 2, 3 and 4 to leverage program number 1. And we -- and you can see from our portfolio how we can do that in the ocular space. We just talked about how we're going to do that in the iNK space as well. And we're also leveraging that into new tissues. So that was -- we've started a program in a neurologic disease using AskBio this time as our collaborator on AAV capabilities, and we'll get into that tissue and try to leverage that in the same way we did in the eye.

Unidentified Analyst

So when you look at EDIT-101, you step back (inaudible) importance of this program to edit some relatively small indications (inaudible). So is this more of the proof-of-concept in establishing your expertise and what you're able to do within the broader vertical of gene editing (inaudible) or something more specific about the indication (inaudible)?

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

Yes, to all of those. And so it's a great place to start because of all the reasons you mentioned. It was also a point mutation in an intron. So if there are unexpected things, some of which came up during the course of the development about possibilities for larger resections, turned out those weren't an issue. But having the point mutation and intron helped with that. Having a disease where we expect the patients to get better, not just prevent a rate of decline, was what you expect to see and LCA10 was a huge advantage, and that's relatively rare and almost unprecedented in the inherited retinal disease space. So there are a lot of advantages to go there. Didn't need to make very much virus, can look at the tissue, really understand the biology multiple ways to measure efficacy, et cetera, et cetera, et cetera. So -- and at the point when we started that program, there were are only 2 tissues you could confidently deliver genetic material to. One was the eye and the other was the liver. Obviously, that's changed since we started that program, but we picked 1 of those 2 because we needed to take out the delivery risk out of the program as well. So there was a lot of it. There was some thought behind how we started, contrary to popular belief.

Unidentified Analyst

What was the motivation in thinking about the partnership with AskBio in the neuro space? As far as, at least, I'm aware, they are pretty early in any neuro program, in the neuromuscular, but (inaudible) CNS programs clinically. So what was sort of -- how did you decide to team with them?

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

It was really about their AAV capability. So they are bring unique stereotypes, they bring manufacturing, they bring a lot of expertise there. People may not realize that AskBio is the company that stood up Bamboo. Bamboo is the DMD asset that Pfizer licensed, and you probably heard about some of the clinical results there. So this is a team that has a lot of experience in the space on the AAV side. And we brought the neuro experience, we didn't ask them for that.

Unidentified Analyst

On that front, when you think about strategically what (inaudible) tools (inaudible), would you expect more of those (inaudible) as part of the smaller sort of technology collaboration that you guys do more of those going forward?

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

I think you'll see a continuum of those. And as I said, we feel that some of these partnerships, even if they are small research collaborations, are important to enabling some of our programs. And so as we need them, we'll add them. We obviously continue to keep active look at even the editing technologies, next-generation editing technologies as well. And so we'll bring in technology as we need it. There's nothing on the near-term horizon that I think we've identified,

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

That's right.

Unidentified Analyst

On the IT front, there's unfortunately not much noise around the same (inaudible). But anything happening behind the scenes which you are thinking about (inaudible).

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

So we believe we have a very strong IP position, particularly as it relates to the foundational Broad-Harvard IP. There is an ongoing interference. We'll likely hear more about that in the -- within the next year or so. There's all kinds of other activity on a global basis as well, but we continue to invest in IP for our programs. And as I said, we think it's important to the field as well as to our own portfolio. And in the end, we'll see how things sort out. But we don't hear as much. You're right. We don't get as many questions these days about IP, but it's important.

Unidentified Analyst

And then the ongoing work around CRISPR, how much innovation is still happening, pushing the boundaries? Obviously, the Cas9 and Cas12A (inaudible). How much is still happening behind the scenes on that front? How early are we (inaudible) really pushing (inaudible) ?

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

The innovative that's going on in the background is really product-focused. So we are working on a few additional bells and whistles, but it's a relatively small fraction of our work now. The vast majority of the scientists are focused on the products and even the ones that are doing what you would refer to as more innovative or blue sky or we can see a path to a product with them. And so if they can make them work, and we think they can, they'll end up in a product very soon, not 5 years from now.

Unidentified Analyst

(inaudible) So I guess, just kind of balance sheet update and cash burden and efficiencies (inaudible).

Michelle Robertson

Sure. So we're well capitalized. We ended 2019 with over $400 million in cash, which is over 2 years of cash for us.

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

Thank you.

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

Thank you.