THOMSON REUTERS

EDITED TRANSCRIPT

Q3 2019 Organovo Holdings Inc Earnings Call

EVENT DATE/TIME: FEBRUARY 07, 2019 / 10:00PM GMT

©2019 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.

CORPORATE PARTICIPANTS

Craig Kussman *Organovo Holdings, Inc. - CFO*
Steve E. Kunszabo *Organovo Holdings, Inc. - VP of IR & Corporate Communications*
Taylor J. Crouch *Organovo Holdings, Inc. - CEO, President & Director*

CONFERENCE CALL PARTICIPANTS

Antonio Eduardo Arce *H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst*
Matthew David Cross *JonesTrading Institutional Services, LLC, Research Division - Research Analyst*
Reni John Benjamin *Raymond James & Associates, Inc., Research Division - Senior Biotechnology Analyst*
Yasmeen Rahimi *Roth Capital Partners, LLC, Research Division - MD & Senior Research Analyst*

PRESENTATION

Operator

Good day, and welcome to the Organovo Holdings Fiscal Third Quarter 2019 Earnings Conference Call. (Operator Instructions) Please note, this event is being recorded.

I would now like to turn the conference over to Mr. Steve Kunszabo, Head of Investor Relations. Please go ahead.

Steve E. Kunszabo *Organovo Holdings, Inc. - VP of IR & Corporate Communications*

Good afternoon, and thanks for joining us. I'd like to welcome you to our fiscal third quarter 2019 earnings call. Joining me on the call this afternoon, our CEO, Taylor Crouch; and our CFO, Craig Kussman. Today's call will begin with a discussion of the 2019 fiscal third quarter results, followed by Q&A.

Before I turn things over to Taylor, I'd like to caution all participants that our call this afternoon may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are statements that are not historical fact and include statements about our future expectations, plans and prospects. Such forward-looking statements are based upon our current beliefs and expectations and are subject to risks, which could cause actual results to differ from the forward-looking statements. Such risks are more fully discussed in our filings with the Securities and Exchange Commission. Our remarks today should be considered in light of such risks. And any forward-looking statements represent our views only as of today. And while we may elect to update forward-looking statements at some point in the future, we specifically disclaim any obligation to do so even if our expectations or views change.

During the call, we'll also be referring to certain supplemental financial measures. These supplemental financial measures are not prepared in accordance with generally accepted accounting principles. Please refer to today's earnings release for a definition of these supplemental financial measures.

With that, let me turn things over to Taylor.

Taylor J. Crouch *Organovo Holdings, Inc. - CEO, President & Director*

Thanks, Steve, and good afternoon, everyone. I'll start by noting that we are once again confirming our key clinical development and operating goals through calendar year 2020. We've also improved our cash -- net cash utilization for the 2019 fiscal year, which, along with our cash on hand, provides us with enough runway to meet our objectives through fiscal 2020.

From an operating perspective, we're making excellent progress in executing the necessary steps for a successful IND and the start of clinical trials. This includes 3 additional proof-of-concept studies, dose ranging studies and all the planning that goes along with chemistry manufacturing and control as well as good manufacturing practices preparation.

Our initial aim is to provide a bridge to transplant with our 3D bioprinted human liver tissue patches for patients with end-stage liver disease, including a select group of patients with inborn errors of metabolism. By implanting our healthy tissue patch in these patients, who often have limited treatment options, we want to restore function or offset the deficiencies and enzyme abnormalities related to a

©2019 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.

specific condition. Ultimately, we hope to delay or reduce the overall

(technical difficulty)

for transplant in these severe unmet disease areas.

As I look ahead to our planned first IND filing in calendar 2020, there are several interim milestones along the way to map our progress. We expect to hold a pre-IND meeting with the FDA in calendar 2019 to focus on the final steps that enable first-in-human trials. We intend to start our IND-enabling toxicity study to support our lead indication in the second half of calendar 2019. The goal of this key study is to determine reasonable safety for potential use in humans. We'll also aim to conduct additional proof-of-concept studies that support the further development of our NovoTissues to treat end-stage liver disease and targeted inborn areas of metabolism.

Finally, we plan to continue pursuing orphan drug designations where it makes sense. We successfully received an orphan drug designation for our NovoTissues treatment of Alpha-1-antitrypsin deficiency in late 2017. Last quarter, we submitted a second orphan drug designation application to the FDA focused on Type 1 Tyrosinemia. In this case, the FDA has responded with questions about the predictability of the animal model we were using and whether this is directly translatable to what we might expect to find in human clinical trials. We're evaluating how we move forward to respond to the FDA's comments, and depending on the outcome of our internal analysis, we may prefer to wait until we have clinical data before further pursuing that particular orphan drug designation.

The potential regulatory designation has no impact on advancing our program toward IND and beyond, and we certainly look forward to availing ourselves of the many other regulatory incentives in the orphan disease, pediatric disease and regenerative medicine space throughout the development process.

We also continue to believe that our NovoTissues will likely have applicability across a number of indications, and as such, we intend to explore the safety profile in patients awaiting transplant who have reached end-stage liver disease from a range of pathways.

To recap our preclinical strategy, we're moving forward with a proof-of-concept enablement plan aimed to demonstrate how our healthy liver tissue patches can potentially address a broad range of target indications. Using the same healthy liver tissue construct, we're already successfully conducting studies in animal models in 2 disease areas. In both A1AT and Type 1 Tyrosinemia, our tissue patch has demonstrated extended retention and robust functionality. In an A1AT model, we've also generated preliminary evidence of [treating] some of the insoluble, misfolded A1AT variants known as globules that are characteristic of the effect of the disease in the liver.

In our Type 1 Tyrosinemia studies, which we published late last year at The Liver Meeting, also known as AASLD, we were able to show an improvement; in the median survival rate of treated animals. Furthermore, we have initiated several new preclinical studies during the fiscal third quarter, including a number of additional proof-of-concept studies, to assess our liver therapeutic tissue performance and to evaluate new treatment modalities. We remain encouraged by our preclinical results, and we'll continue to communicate our ongoing scientific and development progress through publication and major industry events.

As we move closer to our IND filing, our preclinical studies will focus on optimizing our tissue design, dose ranging and proof-of-concept data in new areas. We're also scaling up our liver tissue patches in anticipation of human dosing requirements. And we're opening up new projects in other promising disease areas. In addition, we're advancing our operational capabilities to prepare our chemistry, manufacturing and controls plan. Our final objective is to implement clinical scale manufacturing and quality processes well in advance of our first-in-human trials. These steps encompass a wide range of internal functions, including documentation, equipment and facilities optimization, process validation and the adoption of stringent manufacturing controls.

Of note, we received our first clinical-grade liver late last year through our partnership with the International Institute for the Advancement of Medicine, IIAM. You'll recall that IIAM is one of the world's leading organizations for the procurement of organs used in medical research and the development of therapeutic applications. And we expect to receive a series of these compassionately donated organs over the next year as we prepare for our first IND and the start of human trials.

©2019 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.

While we are advancing our liver therapeutic tissue, we'll also continue opportunistically to pursue revenue-generating projects that leverage our leading 3D bioprinted technology. The Organovo platform includes cell procurement from our Samsara division, bioprinter placement and licensing opportunities and custom service agreements and grants derived from our tissue-generating and modeling capabilities.

We also continue to collaborate with our client on a variety of custom projects that span from exploring liver disease modeling applications to toxicology studies. For example, in the third quarter, 2 large pharma clients came back to us with new projects, both of which focused on evaluating a drug toxicity issue. One of our academic partners, UC San Diego, recently shared promising data on the development of our in vitro nonalcoholic steatohepatitis, NASH, disease model at the NASH-TAG conference in San Francisco a few weeks ago. In short, we induced NASH-like conditions in a 3D bioprinted liver tissue model. These disease tissues were then treated with 2 clinical compounds where a reduction in the disease phenotype, namely fat accumulation, was observed in each case. This is an exciting step in demonstrating the potential value of a human translational model in developing treatment for NASH, a liver disease that affects millions of patients in the U.S. and worldwide. We're grateful to the National Institutes of Health for supporting this groundbreaking work through their grant and to Dr. David Brenner, Vice Chairman for Health Sciences at UC San Diego, who gave a detailed presentation of these results and the potential for advancing drug development in the NASH space.

Our newest board member, Dr. David Shapiro, was also present at Dr. Brenner's presentation. I'm pleased that he is engaging his extensive drug development expertise to help us fine-tune our first-in-human clinical trial design as well as exploring new and innovative ways to develop the functionality of our liver tissue platform.

In closing, we're off to a good start in calendar 2019, with a keen focus on achieving the clinical development milestones that we've laid out over the next 12 to 18 months. I look forward to speaking with you again soon.

With that, I'll turn it over to Craig for a more complete financial review.

Craig Kussman ***Organovo Holdings, Inc. - CFO***

Thanks, Taylor, and good afternoon, everyone. I'll start by reviewing our key profitability and cash flow metrics for the fiscal third quarter and will then summarize our liquidity profile, at-the-market or ATM financing activity and future capital requirements. I'll wrap up my thoughts with a quick recap of our income statement trends.

We posted a fiscal third quarter net loss of $6.4 million, a 22% improvement over the $7.8 million net loss we reported in the year-ago quarter. Similarly, our net cash utilization improved to $4 million versus the $6.5 million in the prior year period. The considerable progress in these bottom line figures is primarily due to a 19% reduction in total costs and expenses related to a streamlining of our operations and R&D programs and, in the case of our net cash utilization, a favorable working capital swing during the quarter.

At the end of December, we had a cash and cash equivalents balance of $35.2 million, which included net proceeds of $1.9 million from the issuance of 1.8 million shares of common stock in ATM offerings. In addition, we've already raised $1.8 million thus far in calendar 2019 through ATM trades. As circumstances and market dynamics permit, we'll continue to use our ATM facility opportunistically to extend the cash runway for the business.

With approximately $41 million of funds available under our ATM facility, we have access to $76 million in capital to carry out our IND development plan. We now forecast an improved net cash utilization rate between $20.5 million and $21.5 million for fiscal year 2019, and we believe we have sufficient funds to meet our operating and capital requirements through fiscal 2020.

The material reduction in our net cash burn versus the last 2 fiscal years will continue to be driven by thoughtful management of our R&D programs and supported by revenue from our commercial opportunities.

Moving now to our income statement and focusing first on operating expenses. Research and development expenses were $3.8 million, a 6% year-over-year decrease, largely resulting from lower employee costs related to our organizational restructuring and the prioritization of our R&D projects. We reported $3.4 million in selling, general and administrative expenses during the fiscal third quarter, a 30%

©2019 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.

year-over-year reduction, primarily due to lower employee expenses.

As we consider our expense trends over the next several quarters, there are 2 important drivers I'd like to reinforce. First, we have the right headcount level today to take us through calendar 2019 to our IND filing with the FDA. We'll continue to rebalance our effort as we gear up our liver therapeutic tissue development, and we'll partner with leading clinical research organizations and topical expert consultants to keep a good balance of fixed versus variable costs. Second, we expect to continue increasing the R&D spend in our therapeutics line of business as we move toward our IND-enabling toxicity study for our lead program and closer to our first IND in 2020.

On the top line, Organovo generated fiscal third quarter total revenue of $0.8 million, which declined 32% from the prior year period. Total year-over-year revenue decreased primarily due to lower grant revenue and fewer active contracts for liver tissue research services in the quarter. As we've shared before, we continue to expect the revenue profile for this part of our business to be unpredictable, partly due to the custom usage of our model.

In conclusion, we're gearing up activity to ready our supply chain, manufacturing processes and quality systems for our first IND in calendar '20 and are moving ahead with the necessary preclinical studies to facilitate a successful pre-IND meeting with the FDA this year. I look forward to updating you on our progress in the months ahead.

With that, I'll turn things back to the operator for the Q&A portion of this afternoon's call.

QUESTIONS AND ANSWERS

Operator

(Operator Instructions) Our first question today comes from Ed Arce with H.C. Wainwright.

Antonio Eduardo Arce ***H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst***

So I have a couple of quick questions here, and perhaps I'll get in the queue for follow-up. Just on your IND filing and the IND-enabling tox that you're looking forward to do later this year, perhaps you could give us an update or confirm previous discussions around the basket approach that you're looking at in terms of this lead program. I know you're thinking about it more holistically and sort of across the inborn errors of metabolism. And how we can think about the types of activities that would be involved to capture a number of those diseases for that work. And then the other question was if you could just give us any updates on the activities around the scaling up and the optimization of that tissue design.

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Sure, Ed. Thanks for those questions. This is Taylor. The -- your first question referred to what we've called our basket approach. And indeed, we feel this is a very interesting and promising way to teed out the capabilities of our tissue. The concept is based on the fact that we are implanting normal, healthy functioning tissue that provides a broad range of function, just like the liver does itself. Our strategy to tapping into this is to start with a broad group of patients who are on the transplant list with end-stage liver disease. These patients have reached that list through a broad variety of disease conditions, including several of inborn areas of metabolism diseases of interest. So we think this is a clever strategy to test our drug in an important arena, the end-stage liver disease, where there really are no other treatment options, but also begin to explore patients with phenotypes that have gotten to that list from a range of disease areas, most importantly, Alpha-1-antitrypsin deficiency, which we would hope to get some very early data on in that first trial. Your second question regarding scale-up. We've done most of our work so far in rodent animal models, which involve, obviously, much smaller tissue patches in order to facilitate transplant into that environment. The human version of the tissue will involve the same structure, composition and bioprinting strategy but just scaled up to a much larger size. And we have had good experience now scaling up our tissues to what we envision to be a human scale level. And we're already talking to surgeons, radiologists and others who are involved in the transplant field about how we would place these patches in a human environment, what approaches make the most sense and how we might model this and get comfortable with these approaches well in advance of our IND filing, all of which we believe will serve us well for launching clinical trials after the IND process.

©2019 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.

Antonio Eduardo Arce ***H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst***

Okay, great. So just a quick follow-up and I'll jump back in the queue. The size of the scale-up, I think you had referred to previously intended for human use as half dollar-sized or something like that?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Yes. We're building up from square base units that we can print out, stitch together in various configurations. The final size of our human patch, we have not publicly disclosed, but you can envision it being somewhere in the range of a dollar bill, perhaps closer to square than rectangular, and basically just comprised of the same units that we're looking -- that we're using in our animal models, just many more of those units.

Operator

Our next question comes from Yasmeen Rahimi with Roth Capital Partners.

Yasmeen Rahimi ***Roth Capital Partners, LLC, Research Division - MD & Senior Research Analyst***

Two questions. Question number one is, given the fact that the liver is a very regenerative tissue, there is quite interpatient variability when it comes to actually bioprinting and implantation, how are you currently modeling and thinking about that before [we're taken] into humans? And then my follow-up question is specifically in regards to in vitro tissue platform. Can you walk me through -- obviously, the last 12 months have been a quite exciting year for the NASH space. Many drug developers have come on to the plate. How many -- if you could quantify, how many additional potential biotech and large pharma partners have you attracted? And what are you projecting as we're moving forward in 2019?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Thanks, Yasmeen. Thanks for those questions. One of the reasons we focused on the liver, in addition to the fact that it's an area of significant unmet need, is that it's a regenerative organ. And at the heart of our patch premise is we put in healthy functioning patches. They ideally will show regeneration in human environment and perhaps help to signal healthier regeneration in the host liver. Both of these are effects that we want to explore in the human models. Unfortunately, in animal models, it's very -- it's not expected to see regeneration. And indeed, so far, we've not seen evidence of it, as we would not have expected. So the encouraging results that we've seen in our A1AT model and in the FAH model that we've used and presented at the -- most recently at the AASLD show very important human functioning of the tissue delay or positive impact on disease onset and, in the case of the FAH model, a actual increase in life expectancy of those animals. But we would expect the regenerative signals only to be seen once we get into the human environment. And that will help us address dosing considerations or answer the question, would one set of implants do the job? Or would one want to do this more than one time, depending on patient outcomes? With regard to our NASH research, we're obviously very, very encouraged that Dr. Brenner, who really is a luminary in the NASH space on a panel, which I believe Dr. [Friedman] was sitting at, presented a lengthy presentation on relevant models for predicting drug functionality in the NASH space in a preclinical environment. And probably more than half of his slides were Organovo slides focused on the progress we've made, demonstrating various drugs performing in our disease tissues and modulating the disease. We also continue to have clients joining us to look at this. Each client typically has asked us to look at a custom version of the model either involving feeding conditions or chemical-induced conditions relevant to what they want to treat with their mechanism of action or even, more recently, starting to look at combination drug strategies. We don't give guidance in terms of number of clients, but we are heartened by the fact that clients that have not worked with us for a couple of years often show up over the transom. We just mentioned 2 such large clients starting to work with us again just this past year, albeit that was in a -- more with a tox question than a NASH question. And we expect to continue to see traction along these routes.

Yasmeen Rahimi ***Roth Capital Partners, LLC, Research Division - MD & Senior Research Analyst***

And if I may ask one last question. Congratulation on the addition of Dr. Shapiro to the board. Will his involvement be mostly on the NASH in vitro tissue capabilities? Or would he also be involved in the sort of development in the therapeutic space?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Thanks. We were very lucky that he actually had deep expertise in both of the areas you mentioned. First and foremost, we wanted to add a talented drug development physician with deep knowledge of liver drug development onto the board. The fact that he has most recently been Chief Medical Officer at Intercept, who's a leader in the NASH space, is obviously quite a nice bonus for us because he's

©2019 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.

also able to look at our in vitro modeling and strategies and provide important insights from having been a client but also having studied this space intensively. So the answer to your question is we look forward to him engaging and providing useful board-level insights on both areas of the business.

Operator

Our next question comes from Ren Benjamin with Raymond James.

Reni John Benjamin ***Raymond James & Associates, Inc., Research Division - Senior Biotechnology Analyst***

I guess, just going back to the manufacturing, Taylor. Can you just provide a little bit more color in terms of, I guess, where you are in terms of the scale-up, obviously, where you hope to be a year from now? What's the -- what's kind of the progress been there? And should we be thinking about different amounts of tissue, if you will, when we're thinking about the rare deficiency-type patients versus those where we might need to be thinking more along the bridge to transplant? Or are they both kind of one and the same? Maybe just a little bit more color on how we should be thinking about that.

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Sure. Thanks, Ren. Well, first, I should mention that we're fortunate that at Organovo, we've spent 10 years developing our abilities to print up viable tissues for a broad range of purposes. And most of that expertise is in printing up liver tissues, which we've used in our in vitro modeling and, increasingly, over the last couple of years in implanting into animals. So our ability to procure, isolate cells through our Samsara subsidiary, expand them, put them through the bioprinting process and print them in various configurations is really a deep skill set shared across a broad range of the scientists here at the company. And therefore, the process of taking this to a larger scale, while it involves a lot of CMC and GMP planning work scientifically, just taps on the exact same skills that we have built in and developed expertise on in-house. In terms of overall volume kinds of metrics, I think we'll start to see some of our operating expense line expand, as Craig had mentioned, as we move toward the clinic related to consumption of materials. But our current bioprinter capabilities and the staff that we have in-house, we believe, is more than sufficient to meet the scale of the clinical trials that we've currently outlined. And you had asked a question sort of relating to dosing. And I would say right now, as I mentioned earlier, we're working with transplant physicians, hepatologists, folks that perform laparoscopic surgery, to answer the question, how much material, i.e., our patches, can we deliver to the liver and in what kinds of configurations? And then we'll work with the FDA to discuss what are meaningful doses and approaches to this. We have fine-tuned these questions, we believe, quite well through expert discussions and, obviously, our preliminary interactions with the FDA over a year ago or -- yes, I guess, it was over a year ago. So we believe that we have a good plan for our dosing. I believe for our initial trials, we expect not to have a huge variability in terms of smaller versus larger doses, but these are things that will work out in the final steps with the FDA.

Reni John Benjamin ***Raymond James & Associates, Inc., Research Division - Senior Biotechnology Analyst***

And can you just help me understand a little bit in terms of the duration of function for the tissues that you've produced in these preclinical models? What's the longest you've been able to follow this function out for? And, I guess, where I'm going with it is, could it one day not necessarily serves the bridge to transplant but really potentially just take over the function and that you could live with that patch?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Sure. So we've published data out to 128 days, which is really quite remarkable given that this is human liver tissue patches transplanted onto the surface of rodent livers. Our hope is that we will see at least that level of functionality in humans. And arguably, one might expect the duration to shoot past that because they'll now be in a much more familiar environment. Certainly, with regard to bridge to transplantation, an effect measured in months starts to have a really meaningful benefit for patients waiting in the shortage of livers, many of whom never actually get to a transplant. So we believe, in that environment, a handful of months is meaningful. We would hope to see what you've suggested, which is a longer-term effect, maybe even the possibility of obviating the need for a transplant. But these are all things that we'll just have to wait and see what transpires in our human clinical trials. The good news is we've got evidence that would suggest that the duration of effect that we're seeing now would be meaningful clinically and with regard to commercial opportunity in these patients.

©2019 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.

Reni John Benjamin ***Raymond James & Associates, Inc., Research Division - Senior Biotechnology Analyst***

Got it. Maybe just one final one from me. I was actually a little bit surprised to hear that the FDA pushed back on the model that you're using for hereditary tyrosinemia. Have -- I guess, have you gone back to kind of look at all the other models that you're doing to make sure that those would, in fact, be acceptable by the FDA? I think you mentioned that there are quite a few new preclinical studies that have been initiated. Can you just provide us with a little bit of color as to what you hope to learn from those?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Sure. So there's 2 components to your question. I'm going to start first with the fact that we met with the FDA very extensively in our pre-pre-IND meeting based on a briefing book that had an extensive animal data of the kind that we submitted for this orphan drug designation application. And at that meeting, the FDA was very encouraging about the animal modeling approaches we were using and the path that we felt would take us to the clinic based on that data. So the big take-home is that we're building a strategy to the clinic on solid animal modeling work. Very separate from that, the FDA chooses to grant orphan drug designations and certain benefits to the cost and time lines for those recipients. And the threshold they're using there, primarily, they like to see the space on human data. We were lucky, our first time around, that we had a compelling set of data and the Alpha-1-antitrypsin model, such that they granted us designation upfront. This is the same tissue that we used in the second animal model, which is not exactly replicating the human disease condition but provides good evidence of the kinds of functions we would want to argue for going into clinical trials in that area. That's why I said there are questions and concerns about granting the designation now have really no bearing on the animal evidence or the applicability towards an IND. They would just like to see more data specific to the human disease before they grant the orphan designation. And we'll certainly have more bites of that apple. And we may choose to go back and challenge them or provide more data now. Or just as likely, we may sit on this and just wait until we're in the clinic and go back for that particular designation.

Operator

(Operator Instructions) Our next question comes from Matthew Cross with JonesTrading.

Matthew David Cross ***JonesTrading Institutional Services, LLC, Research Division - Research Analyst***

Just first off, I wanted to ask -- I don't want to make too much out of this response from the FDA regarding the designation for tyrosinemia, but reaching back to Ed's question on this basket approach to an IND, does this response from the FDA kind of give you any pause as far as the strategy, with the agency kind of signaling that these liver IEMs will be viewed as having discrete concerns to address?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Matt, I think that's a great question. And I do think that our discussions in the pre-IND -- primarily in pre-IND meeting will revolve around how quickly do we branch out, what preclinical and to what degree do separate clinical plans need to be developed for each IEM. One reason why we're excited about the strategy of going in, looking at end-stage liver disease is that -- and we've had several transplant surgeons tell us or hepatologists tell us that once a patient's put on a liver transplant list, the focus is on the functionality of the liver and not how they got there. And indeed, in many cases, they don't even know. I mean, they haven't even diagnosed whether the patient maybe has one of these IEMs as the underlying cause that got them to this point. That said, we believe we'll be exposed to patients who've reached the list from a range of indications that have led to cirrhosis and diminished function. And this is going to give us experience in those patients and allow us to build up arguments for expanding our clinical investigation in those patient groups. In parallel, we -- and as I mentioned, we are starting a series of proof-of-concept studies in other areas as well as drilling into areas of interest in and around that space I just described to provide as much data to the FDA as possible, showing the functionality and the nuances of how our tissues respond in various disease states in these animal models. So we believe we'll be armed with a great set of data to convince the FDA that this is a strategy that can quickly branch into multiple opportunities.

Matthew David Cross ***JonesTrading Institutional Services, LLC, Research Division - Research Analyst***

Got it. Okay. I appreciate the detail around the directions. And you actually kind of led into my next question, which was, I was hoping you might be able to give us a little bit more detail around those proof-of-concept studies you're running in these collection of animal models. Basically, I'm trying to understand what conclusions you're hoping to reach from these studies and how that may slot into the pre-IND process with enough time still to prepare before CMC review, tox studies and the like or if you really envision these as kind of preclinical studies as avenues for future expansion post-IND filing to move beyond these liver IEMs.

©2019 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.

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Right. Well, there's probably 2 categories in general to look at. One is showing the broad range of functionality of our base tissue in a range of conditions. So those conditions can involve sicker animals, placement of the tissues in different positions and different doses in those animals, different surgical conditions one might use. This is all aimed at providing the surgeon or the treating physician a broad range of avenues to explore for inserting healthy, functioning tissue into their specific patient. And that's important because it's a creative group, and there's a lot of heterogeneity among patients, liver shape and conditions. And we want to have as much functional clarity and examples as possible shown in our proof-of-concept. The other area is to explore the drug -- the patch in animals that have been purposely bred deficient of each of the various enzymes -- missing enzymes in these inborn conditions and explore in each condition how our patch might provide an effect. We're able to measure the human production of these enzymes in all of our animals in certain conditions, but obviously, you'd like to see how it would perform in an animal that's completely deficient of the animal version of that enzyme. All of these gets pretty tricky and probably gets a little bit back to the question the FDA asked us on a particular animal model we were using to show the Type 1 Tyrosinemia potential effect. You can't create that human disease the exact way we would want to study it in animals. So all you can do is come at it around the edges, the way I've described. And so we're not concerned about their response. We knew that we were honing in on evidence that should allow the FDA to conclude, yes, this is a good basis for studying the drug in humans. The group that determines ODD grants or valuations is separate from that. And they (inaudible) prefer to have human data. We are heartened by the fact that at the AASLD meeting last November in the rare disease panel, we were one of the very few preclinical companies asked to present amongst a number of luminaries presenting cutting-edge therapies late in the clinic are already commercially available. And we were there presenting our preclinical data on Type 1 Tyrosinemia. And it was an extremely enthusiastic response from that audience of experts. And so again, I think showing the potential ultimately to create enough confidence that we now have a benefit worth exploring in humans, that's what we're trying to shore up with as much proof-of-concept data as possible going into these FDA meetings.

Operator

Our next question is a follow-on from Ed Arce with H.C. Wainwright.

Antonio Eduardo Arce ***H.C. Wainwright & Co, LLC, Research Division - MD of Equity Research & Senior Healthcare Analyst***

So I just wanted to ask a question about this announcement you recently entered into with IIAM. It sounds like this clinical sourcing agreement with them is at least in part to source whole organs. And so I'm -- and perhaps cells and tissues as well, but you mentioned the whole organ recently received. And so I'm wondering, how does that perhaps complement the liver cells, the liver cells that you get from your own internal division, Samsara, the [biolink] that you use and sort of the stem cells also that you use? How does all of that work together, especially as you think about continuing to optimize your tissue design and scaling up?

Taylor J. Crouch ***Organovo Holdings, Inc. - CEO, President & Director***

Sure, Ed. That's an important question. So let me start with IIAM. They are a not-for-profit group that works with, and I believe it's the 58 organ procurement agencies spread across the 50 U.S. states, these group of organ procurement organizations, or OPOs, are the groups that provide organs for the transplant world here in the United States. Those organs that are not deemed applicable for various reasons, which could just be timing or a lack of patients or other reasons, those organs that are not deemed applicable for a transplant then are directed for research use. And IIAM is at the forefront of procuring these organs and getting them to where the research causes. And our announcement was quite strategic because we are delighted to be working with them as the largest group focused on making sure every compassionately donated organ has the most potential to affect human condition. And the fact that we now will have a steady supply of those is really quite an endorsement of what we're doing here and the potential of how we might affect these serious diseases. What we do is we isolate cells from donated organs. Those cells are the building blocks that our Samsara group uses to provide to the research community and increasingly now to our own tissue platform. And those tissues, comprised of these cells that ultimately have come from high-quality donated organs that are not -- that cannot be used for transplant but certainly can be saved and repurposed and, ideally, through our model, brought back to treating patients, really provide kind of a second cycle of life in this very important transplant field. So hopefully, that explains the significance of that relationship.

Operator

This concludes the question-and-answer session as well as the conference. Thank you for attending today's presentation, and you may now disconnect.

©2019 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.

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 Briefs 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 BRIEFS REFLECTS THOMSON REUTERS'S SUBJECTIVE CONDENSED PARAPHRASE OF THE APPLICABLE COMPANY'S CONFERENCE CALL AND 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 BRIEF. 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.

©2019 Thomson Reuters. All Rights Reserved.

©2019 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.