**Organovo Holdings, Inc. (Q2 Earnings)**
**November 8, 2018**

Corporate Speakers

- Steve Kunszabo; Organovo Holdings, Inc.; VP of Investor Relations & Corporate Communications
- Taylor Crouch; Organovo Holdings, Inc.; CEO, President & Director
- Craig Kussman; Organovo Holdings, Inc.; CFO
- Steven Hughes; Organovo Holdings, Inc.; Chief Medical Officer

Participants

- Ren Benjamin; Raymond James & Associates, Inc.; Analyst
- Ed Arce; H.C. Wainwright & Co, LLC; Analyst
- Matthew Cross; JonesTrading Institutional Services, LLC; Analyst

## PRESENTATION

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

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

Steve Kunszabo: Good afternoon, and thanks for joining us. I would like to welcome you to our fiscal second quarter 2019 earnings call. Joining me on the call this afternoon, our CEO, Taylor Crouch; our CFO, Craig Kussman; and our Chief Medical Officer, Steven Hughes.

Today's call will begin with the discussion of the 2019 fiscal second 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 facts 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. 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 reviews 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 definition of these supplemental financial measures.

With that, let me turn things over to Taylor.

Taylor Crouch: Thanks, Steve, and good afternoon, everyone. I'll begin by highlighting that we're on track to achieve the key clinical development and operating goals that we've outlined through calendar 2020.

We're moving ahead with additional relevant preclinical studies to broaden our proof-of-concept data across a range of disease conditions. We are also engaging with trusted advisers with expertise in liver disease, surgical techniques and regenerative medicine regulatory strategies to fine-tune our fastest-effective path to the clinic.

Our aim is clear, treating a spectrum of pediatric and adult liver diseases with our 3D bioprinted human liver tissue patches. We are targeting rare and often life-threatening conditions, which currently have limited treatment options and the shortage of organs available for full liver transplants leaves many patients in desperate need of new potential therapies.

To start, we focused on animal models of single mutation inborn errors of metabolism, including Alpha-1-antitrypsin deficiency, A1AT, and Type 1 Tyrosinemia. Our objective in implanting a healthy tissue is to restore function or offset the deficiency of a specific enzyme abnormality. Ultimately, by conducting successful future studies, we also hope to show that our liver therapeutic tissue would delay or reduce the need for a transplant.

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 believe that our development of a healthy therapeutic liver tissue patch can create -- can treat a broad range of rare disease indications. To that end, we're pursuing a second orphan drug designation with the FDA. We expect news on this additional orphan designation in the first half of calendar 2019.

We also expect to hold a pre-IND meeting with the FDA in calendar 2019 to focus on the final steps that enable human clinical trials in A1AT patients and others awaiting transplant.

And finally, we intend to start our IND-enabling toxicity study to support multiple indications, including Alpha-1-antitrypsin deficiency in the second half of calendar 2019.

In addition, we will continue to conduct proof-of-concept animal studies in multiple rare diseases. The goal of these important studies is to determine reasonable safety and

potential use in humans and to identify baseline efficacy activity that justifies further commercial development.

As I noted earlier, we're moving forward with this strategy that allows our healthy NovoTissues to potentially address a broad range of target indications. Using the same healthy tissue construct, we've now successfully conducted early studies in established animal models in two disease areas. In both A1AT and Type 1 Tyrosinemia, our tissue patch has demonstrated extended retention and robust functionality, including production of expected human enzymes missing in these inborn errors of metabolism deficiencies.

In an A1AT model, we've also generated preliminary evidence of reducing some of the insoluble, misfolded A1AT variance, known as globules, that are characteristic of the effect of this disease on the liver, and which overtime can lead to liver failure.

In our Type 1 Tyrosinemia studies, which we've just published for next week's key industry conference, The Liver Meeting up in San Francisco, we were able to show an improvement in the median survival rate of treated animals. We remain encouraged by our preclinical results, and we'll continue to communicate our ongoing scientific and development progress through publications in major industry events.

Our therapeutic solutions not only offer the promise of significant patient impact, but also represent an attractive revenue opportunity for Organovo. By focusing on a group of orphan liver diseases that includes inborn errors of metabolism and end-stage liver disease, we are participating in a significant market that we believe has greater than $1 billion of potential.

While we are advancing our liver therapeutic tissue, we'll also continue opportunistically to pursue revenue-generating projects that leverage our 3D bioprinting technology.

The Organovo platform spanned 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.

Capitalizing on these expertise and procuring and characterizing specialized human liver cells, Samsara recently launched a new product, offering an RNA-sequencing data library with matched sets of human livers tissues and cells from a range of healthy and NASH disease donors. This solution enables customers to mine data for the discovery and validation of disease and cell-specific markers in a cost-effective manner.

We also continue to collaborate with our clients on a variety of custom projects, including NASH, that span from liver disease modeling applications and toxicology studies.

In closing, we've made good progress over the last few months as we move closer to our key clinical development milestones. Culminating with an IND for our liver therapeutic tissue in calendar 2020. I look forward to sharing our progress in the months ahead.

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

Craig Kussman: Thanks, Taylor, and good afternoon, everyone. I'll start by summarizing our key profitability and cash flow metrics for the fiscal second quarter, and we'll then review our liquidity profile at-the-market, or ATM financing strategy and future capital requirements. I'll wrap up my thoughts with a quick recap of our income statement trends.

We posted a fiscal second quarter net loss of $5.8 million, a 38% improvement over the $9.5 million net loss we reported in the year-ago quarter. Similarly, our net cash utilization improved to $4.3 million versus the $8.3 million in the prior-year period.

The substantial progress in these bottom line figures is primarily due to a 36% reduction in total costs and expenses related to a streamlining of our operations and R&D programs.

At the end of September, we had a cash and cash equivalents balance of $37.4 million, which included net proceeds of $2.1 million from the issuance of 1.7 million shares of common stock in ATM offerings. As circumstances and market dynamics permit, we'll continue to use our ATM facility opportunistically to extend the cash runway for the business as we've successfully done throughout calendar 2018.

With approximately $45 million of funds available under our ATM facility, we have access to $82 million in capital to carry out our IND development plans. We continue to forecast a net cash utilization rate between $22 million and $24 million for next fiscal year 2019, and we believe, we have sufficient funds to meet our operating and capital requirements through fiscal 2020. In fact, it's looking increasingly likely that our net cash utilization rate will come in at the bottom end of this range. The material reduction in our net cash burn versus the last two 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 & development expenses were $3.2 million, a 36% year-over-year decline, primarily resulting from lower employee and lab supply costs related to our organizational restructuring and the prioritization of our R&D projects.

We reported $3.6 million in selling, general and administrative expenses during the fiscal second quarter, a 37% year-over-year decrease, largely due to lower employee and noncash stock-based compensation expenses, as we continue to streamline our operations during the second quarter.

As we consider our expense trends over the next several quarters, there are two important drivers to keep in mind: first, we have the right headcount level today to take us through the calendar 2019 and into our pre-IND meeting 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 do expect to ramp up the R&D spend in our therapeutics line of business, as we began our IND-enabling toxicity study in multiple indications and move closer to our first IND in 2020 and beyond.

On the top line, Organovo generated fiscal second quarter total revenue of $0.9 million, which decreased 30% from the prior-year period, but was up 37% sequentially.

Total year-over-year revenue declined due to 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 and our not forecasting any specific total revenue targets for fiscal 2019.

In wrapping up, we remain squarely focused on achieving the clinical development milestones we've laid out over the next 12 to 24 months. We continue to generate promising scientific data from our preclinical studies such as the work we'll share next week at The Liver Meeting around Type 1 Tyrosinemia, and our first IND in calendar 2020 is starting to come into view.

I look forward to speaking with you again soon. 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) And our first question today will come from Ren Benjamin with Raymond James.

Ren Benjamin: Congrats on the progress, especially, as you tighten up the timelines in regards to getting ready to get into the clinic. I guess, the couple of questions for me. One, Taylor, can you talk a little bit or try to see if you can get a little bit more specific regarding this meeting with the FDA in 2019. Just based on your comments, I would think that the meeting would likely take place in the first half of 2019 and based on those discussions, you'd be conducting the IND-enabling studies in the second half of 2019. Does that sound right? Or is there any color you can provide?

Taylor Crouch: Well, we haven't given specific guidance as to when the meeting would occur. So I'd say two things: one, because we afforded ourselves the opportunity to meet the FDA in the pre-pre-IND process, this summer, we've already gotten a pretty extensive readout for how appropriate our strategy is heading towards the IND process. And that's why we've been able to affirm our guidance several times since that call.

With regard to the pre-IND meeting in next year, this will be kind of our final checkpoint to make sure that our animal studies and CMC strategies are buttoning down appropriately to head into the formal IND process in the following year. And as we signaled, we do expect to begin our IND-enabling tox study in the second half of next

year. So you can start to do them and -- as to where this meeting will fall in the calendar year.

Ren Benjamin: Got it. Okay, that helps out. And then outside some of, sort of, the preclinical data that's going to be generating in these IND-enabling studies, which, I assume, will also contain nonhuman primates. Can you talk a little bit about any progress or any thoughts in regards to generating larger amounts of tissue? And so increasing larger tissue size and potentially at the same time, trying to drive down costs and just, sort of, your thoughts in regards to those two items.

Taylor Crouch: Sure. With regard to animal studies, and we've not revealed our strategy externally as to which models and which animals we'll be studying in. We do have good feedback, as I mentioned, from the FDA as to what they would view as an appropriate backdrop for demonstrating efficacy prior to going into humans.

With regard to your scale up question, couple of points: one, the advantage we have at Organovo after 10 years of perfecting our technology platform around 3D bioprinting is that we understand scale -- we believe, quite affectively, the patches that we're using in animals represent a sizable scale up over the in vitro patches -- in vitro tissues that we used in our commercial modeling business. We've also begun the process of scaling up patches from animal size to human dose size and what I can say is that, that process of printing out a suitable patch unit for humans can still be measured in minutes rather than hours or more, because of the speed and efficiency of our platform.

With regard to the cost of raw materials, since our building blocks are human cells, these costs are relatively fixed in terms of the inputs. But our manufacturing process, we believe, is about as fine-tuned as one could hope to have for approaching human clinical trials and even giving us a pretty good runway of visibility as to how we might scale up ultimately for commercial manufacturing.

Ren Benjamin: Got it. And just can you remind me, because I'm going back here and going off memory, but the size of tissue, maximum size of tissue that you've been able to generate so far, is it still, sort of, that dollar bill size that we're thinking about? Or have we gone past that now?

Taylor Crouch: Well, we've been printing in various sizes up to, let's say, portions of a dollar bill. As we think about the geography of the liver and talk to surgical experts and the process of transplanting these patches -- or implanting these patches, we've begin this hone-in on what scale the unit dose, if you will, should be to provide the maximum flexibility and efficiency from a surgical perspective.

From our perspective, the end-size in scale of these patches is not something we're too concerned about, again, just because of our experience in printing under -- printing multiple, multiple shapes, multiple dimensions and multiple scales for various purposes.

Operator: Next question will come from Ed Arce with H.C. Wainwright.

Ed Arce: A couple for me. So first, Taylor, you had mentioned in your prepared remarks, just at a top level, some of the results you've seen so far in your animal models, in both A1AT and HT-1, describing the robust functionality that the patches have delivered so far, including with -- aligned with the reengagement of the proper enzymes. One question around that and I know this is specific to HT-1, I believe, could you discuss a little bit further what you had mentioned earlier about the globules?

Taylor Crouch: Okay. Ed, so the comment on the globules actually relates to Alpha-1-antitrypsin deficiency. The misfolded proteins get trapped inside these cells in the liver and slowly clog the function leading to what they're called, globules, and ultimately, leading to a significant deterioration in liver function, such that some of these patients who may have survived childhood and the lung manifestations of the disease end up with a declining liver and in need to have a transplant. And we were heartened by the fact that in our animal studies, we noted that the area adjacent to our patches in the host animals, which normally would be a clog with these modules, was showing a clearing of modules at least to a certain depth beneath the patches. And that could be a very interesting finding that we'll want to explore further.

Ed Arce: Okay, great. The other question was just, I think, we discussed this briefly actually two, three weeks ago, but just to further clarify for my own benefit. There are two indications that you had probably with both of them a year or so publically pursuing. I believe, you consider that as part of the one IND that you're looking to submit in 2020? And thus, you consider a second indication separate from those two? Is that correct?

Taylor Crouch: Yes. To clarify, the IND, we believe, will give us permission to take this patch strategy into patients into our initial study, which will likely take place in patients on a transplant list, who've reached that condition through multiple disease drivers, including, for instance, Alpha-1-antitrypsin disease. So in other words, we hope and expect that our IND will open up an avenue to developing and testing patients with multiple disease types with the same fundamental patch product.

Ed Arce: Okay, got it. One last quick one, if I may. Since you had mentioned just now the ability that you have, not only substantial size now with the patches, but also multiple different shapes and dimensions. I was wondering if you consider in any one given patient the ability to customize the therapeutic with multiple patches before a given organ?

Taylor Crouch: Well, so I want to maybe correct or clarify one thing. By shape, I'm primarily focused on geometric shapes, squares and the like of varying thicknesses. But primarily, relatively, thin in the dimension. The reason that we think about custom shapes is that we're talking about the intricate contours of various organs. And as we look at various organs strategies, including the liver, we'll explore whether there is certain geometries that best fit the treatment strategy.

With regard to custom patient solutions, there are few aspects to consider. We do expect to do something and can do just-in-time printing for a given patient, as they approach the need for a patch. So that would be, you could say, a patient-specific strategy. However, we would use standardized approaches for that printing and the only thing we might vary our certain cell compatibility types specific to that patient that we may need to adjust to. But the actual dimensions of the patch, all of the manufacturing conditions, et cetera, would all be standardized. So that we're not creating a custom on the spot product for each patient.

And that to get back to Reni's point, that's also partly to just try and manage our cost, but also manage the speed with which we can get these remarkable solutions to patients in a just-in-time fashion.

Operator: (Operator Instructions) And our next question comes from Matthew Cross with JonesTrading.

Matthew Cross: Just wanted to follow a similar avenue of question to Ed as far as your pursuit of another rare or orphan disease to a designation. I am just a bit curious why tyrosinemia hasn't been declared as a second indication just based on the encouraging data that you've already presented in those models? And wondering, how we should be thinking about that indication given the upcoming data at AASLD and while other work is in process?

Taylor Crouch: That's a great question and perhaps the reason why we've spoken in nuance about nominating an indication versus the ones that are slowly being validated in our proof-of-concept study is more about the sequence of when we would being seeking patients in our clinical development program. Type 1 Tyrosinemia plays out largely in a pediatric population, and we know initially in our clinical development plan that we will need to focus our patient treatment activities, certainly [in the first] safety study in adult patient population.

So at the moment, we're not giving guidance as to when we might begin to aim for pediatric populations and the best disease fits for our patch in those populations. But we do plan over the next year to give more and more color about how we see indications unfolding as we move into our clinical development plan and will help you all with your modeling as to how we see these opportunities in end markets matching up with our patch strategies.

Matthew Cross: Got it. Okay, that's very helpful. And I can't blame you for wanting to see how these other proof-of-concept animal studies play out before making that decision.

Second one I had was just, are you able to give any more clarity around what you're hoping to receive guidance on from the FDA at this upcoming meeting in order to move forward towards final pre-IND work? I guess, obviously, there's a technology that could overall generate a lot of unique data the agency may be hoping to see prior to clearance

for human study relative to other kinds of therapeutics. So I was wondering if you could comment on what you expect this may entail as we try to follow the path to a 2020 IND? Or is it something more indication-specific?

Taylor Crouch: Sure. So I'll start with that and may be Steve would also like to jump in. There are some general aspects of approaching an IND in regenerative medicine that are moving very quickly. The FDA as well as our industry is rapidly trying to develop standard approaches to testing and preparing documentation to enter into the space.

And so one of the goals at the pre-pre-IND meeting this year, the pre-IND meeting next year and obviously, ultimately, the IND is to just check in with the FDA on whether we're still all on the same track, are there nuances that are evolving that we should be aware of. But generally, we've laid out our strategy with the FDA. We're blocking and tackling on that strategy, and I'll turn it to Steve, who may have some more specific points about how he sees the discussions, the topics evolving for the pre-IND meeting.

Steven Hughes: Thanks, Taylor. The IND -- I'll may be frame it slightly before answering the question, which is a -- the pre-IND meetings are of finite length. So we don't have an infinite amount of time to discuss with FDA. I know a range of potential questions that we could ask and we -- in multiple areas, including the animal days that we have to support, the clinical work that we wish to do, our manufacturing process and what we need to document, et cetera, in order to satisfy the FDA's requirements to begin human testing. And also, the design of our clinical trials, the endpoints of our clinical trials and the patient -- target patient population that we're planning to go into for our first-in-human study.

So there are multiple potential areas where we could engage the FDA in a dialogue to get their feedback. And what we're doing is going through the process of mapping out the questions and why there's areas of uncertainty and prioritizing those questions into ones, which we would like to ask within the meeting.

So at this point, I can't give you specifics of these are the questions that we are going to ask the FDA, but they will be within the general areas broadly of the CMC side, the clinical development plan and the animal data that we have to support the indications that we propose to go into for our first-in-human study.

Matthew Cross: Understood. Okay. Thanks, Steve and Taylor, I presume you guys have just made some points for me on the, kind of, overall strategy and look forward to seeing your presentation at AASLD.

Operator: And this will conclude our question-and-answer session as well as today's conference. We thank you for attending the presentation and you may disconnect your lines at this time.