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Arcturus Therapeutics

Third Quarter 2019 Earnings Call

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CORPORATE PARTICIPANTS

Neda Safarzadeh, *Head of Investor Relations, Public Relations, and Marketing*

Joseph Payne, *President and Chief Executive Officer, Director*

Andrew Sassine, *Chief Financial Officer, Director*

Padmanabh Chivukula, *Chief Scientific Officer, Chief Operating Officer*

CONFERENCE CALL PARTICIPANTS

Wangzhi Li, *Ladenburg*

Ed Arce, *H. C. Wainwright*

Kumar Raja, *Brookline Capital Markets*

PRESENTATION

Operator:

Good day and welcome to the Arcturus Therapeutics Third Quarter 2019 Earnings Conference Call. Today's conference is being recorded.

At this time, I would like to turn the conference over to Neda Safarzadeh, Director, Head of Investor Relations, please go ahead ma'am.

Neda Safarzadeh:

Thank you, Operator, and good afternoon, everyone. Thank you for joining Arcturus's Earnings Conference Call. We are excited for this opportunity to discuss the Company's third quarter 2019 operating results.

We are joined today by Joseph Payne, our President and CEO, and Andy Sassine, our CFO. Dr. Pad Chivukula, our CSO and COO, is also on the line, and will be available to address questions during the Q&A session. Joe will kick off the call with a high-level review of Arcturus. Next, we will discuss first quarter financial results and recap recent developments towards strengthening the Company's balance sheet. Finally, we will open the call for your questions.

Before we begin, I would like to remind everyone that, except for statements of historical fact, the statements made by Management and any responses to the questions on this conference call constitute forward-looking statements that involve substantial risks and uncertainties for purposes of the Safe Harbor provided by the Private Securities Litigation Reform Act of 1995. Any statement, other than statement of historical fact, included in this communication, including those regarding strategies, future operations, the

status of preclinical and clinical development programs, the status of clinical development programs and the Company's future cash and financial position, are forward-looking statements. Actual results and performance could differ materially from those projected in any forward-looking statements, as a result of many factors, including without limitation an inability to develop and market product candidates, unexpected clinical results, and general market conditions that may prevent such achievements and performance.

Such statements are based on Management's current expectations and involve risks and uncertainties including those discussed under the heading Risk Factors in Arcturus's annual report on Form 10-K for the fiscal year ended December 31, 2018, filed with the SEC on March 31, 2019, and in subsequent filings with or submissions to the SEC. Except as otherwise required by law, Arcturus disclaims any intention or obligation to update or revise any forward-looking statements, which speak only as of the date they were made, whether as a result of new information, future circumstances, or otherwise.

I will now turn the call over to Joe.

Joseph Payne:

Great, thank you, Neda. Good afternoon and thank you for joining us on the call today for our Third Quarter Financial Results and Corporate Update Conference Call. We are very enthusiastic about the potential that our messenger RNA medicines have to treat patients with serious, challenging, life-threatening diseases.

Today we are pleased to discuss with you the progress of our pipeline development programs, our platforms' recent business developments, and our plans to ensure our continued success.

I think it would be worthwhile to begin by reiterating what it is we do and what drives all of us here at Arcturus. We are a leading messenger RNA medicines company, focused on the development of therapies that target a wide range of illnesses, such as orphan diseases, liver and respiratory diseases, cancer, and infectious diseases that have a significant unmet medical need. We are dedicated to finding solutions for these diseases where there are no cures or effective treatments, including our most advanced programs, which include internal pipeline therapies to treat ornithine transcarbamylase deficiency or OTCD, cystic fibrosis, and externally partnered platform programs such as glycogen storage disease type 3 or GSD3 with our collaboration partner Ultragenyx.

Well, 2020 aims to be an exciting year for the field of messenger RNA therapeutics, and Arcturus is fortunate to be a leader in intravenously delivered mRNA therapeutics. On that note, I will now highlight some of our key developments for our flagship program, ARCT-810, an intravenously delivered mRNA medicine for OTC deficiency.

I am pleased to report that we remain on track to file an investigational new drug application with the FDA in the first quarter of 2020. We have successfully completed multiple GMP-manufactured batches of drug substance. These batches of ARCT-810 drug substance have passed release criteria and are intended for use in human trials.

The in life phase of the preclinical IND enabling safety studies to support first in human clinical trials has been completed. Please note that Arcturus still has full commercial rights to ARCT-810. The product has been granted orphan drug designation in OTC by the FDA. We have a clinical plan in place for this program, and we intend to publicly share more details pertaining to the ARCT-810 clinical plan early next year.

OTCD is the most common urea cycle disorder. Urea cycle disorders make it difficult for afflicted patients to remove toxic waste products as proteins are digested. OTC deficiency is caused by mutations in the OTC gene, which leads to a nonfunctional or deficient OTC enzyme. ARCT-810 utilizes our Lunar lipid-mediated delivery platform designed to effectively deliver OTC messenger RNA to liver cells and

enable the patient to naturally produce healthy functional OTC enzymes in their own liver cells. By intervening directly in the underlying disease process, ARCT-810 has the potential to be a significant new messenger RNA therapy for patients afflicted with this disease, which can often cause neurological damage and severe damage to the liver, requiring a transplant.

Now on to our cystic fibrosis program, Lunar CF.

In the third quarter of this year we announced that the Cystic Fibrosis Foundation increased its commitment to $15 million, in conjunction with an amended agreement to advance Lunar CF, a novel messenger RNA therapeutic formulated with our Lunar delivery technology. We have recently received the first payment of $4 million from this commitment, and our collaboration with the Cystic Fibrosis Foundation began in 2017. The goal of the multi-year program is to create mRNA therapies to treat people with cystic fibrosis and develop methods to deliver RNA to cells in the lung, and file an investigational new drug application for a therapeutic candidate. Our goal is to submit an IND application for our first CF candidate in 2021, and to commence trials that year.

As part of our efforts to advance our CF program, we plan to hire additional leadership with significant pulmonary therapeutic experience to lead and manage our lung franchise to ensure that we fully capitalize on this significant opportunity for the Company.

Another value driver for Arcturus is our platform. Our expanding technology platform continues to show promise. Earlier this morning, we announced the expansion of our technology platform to include self-replicating RNA for human and animal health vaccine applications. We call this technology Starr or S-T-A-R-R, which stands for self-transcribing and replicating RNA. When Starr technology, or this self-replicating RNA technology, when it's delivered into the cell, it can generate a protective immune response or drive therapeutic protein expression to potentially prevent or treat a variety of diseases. The self-replicating RNA-based therapeutic vaccine triggers rapid and immediate antigen expression within host cells, and a stronger T cell response. This combination of Starr technology, formulated in our biodegradable Lunar delivery system, is expected to more comfortably provide a longer-lasting RNA vaccine, and thus lowering dose requirements compared to traditional RNA-based vaccines. They can be delivered in a softer more biodegradable manner that is less inflammatory. Simply said, the Starr technology platform combines self-replicating RNA with Lunar, and that's Arcturus's lipid-mediated nanoparticle delivery system, into a novel single source solution for partners.

Now just some recent developments regarding our leadership and Management team. In August we announced that Doctor Edward Holmes joined our Board of Directors, bringing additional experience and expertise to our highly qualified Board. We are delighted to have him on the Board and we look forward to his contributions and working with him.

I will now turn the call over to Andy, who will provide the details of our financial results for the quarter.

Andrew Sassine:

Thank you, Joe, and good afternoon, everyone.

During the third quarter, the Company raised $23 million in gross proceeds from institutional investors at $11.50 a share. This is the first time Arcturus has raised money from institutional investors, and we are excited about the new institutions, who are among the largest and most successful biotech funds in the industry. We are also grateful for the support we received from Ultragenyx, our largest strategic partner and shareholder, to complete this raise to help fund our future pipeline of messenger RNA therapeutics.

We issued a press release earlier today that included financial statements for the third quarter ended September 30, 2019, which I will briefly summarize. Collaboration revenue was $3.3 million during the quarter ended September 30, 2019, compared to $3.4 million in the prior year third quarter. Operating expenses were $10.9 million in the quarter ended September 30, 2019, compared to $7.8 million in the prior year quarter. Net loss for the quarter ended September 30, 2019, was approximately $7.4 million or $0.56 per basic and diluted shares, compared to a net loss of $4.3 million or $0.42 per basic and diluted share in the prior year period.

I will now provide a summary on financial results for the nine months ended September 30, 2019. Collaboration revenues were $17.8 million for this period, compared to $8.2 million in the nine months ended September 30, 2018. Operating expenses were $32.5 million in the nine months ended September 30, 2019, compared to $29.3 million in the prior year period. Net loss for the nine months ended September 30, 2019 was approximately $15 million or $1.18 per basic and diluted share, compared with a net loss of $20.8 million or $2.07 per share in the prior year period.

At September 30, 2019, Arcturus had cash and cash equivalents totaling $74.2 million, compared to cash and cash equivalents of $36.7 million at December 31, 2018. Subsequent to the end of the fiscal quarter, we received approximately $7 million as a result of increasing our bank facility and an insurance settlement. This provides adequate funding to support the Company's current programs through at least the first quarter of 2021.

Joe, I will now turn the call back over to you.

Joseph Payne:

All right, thanks, Andy.

In summary, everyone, we are continuing to advance our pipeline programs that target diseases with significant unmet medical needs in large potential markets, focusing on life-threatening rare diseases where there is no cure.

We continue to expand our chemistry delivery and manufacturing technology platform, with the recent addition of self-replicating RNA or Starr technology for human and animal health vaccine applications.

We are fortunate to have strong collaborations that have the potential to bring in more than $1 billion in milestone and royalty payments to Arcturus.

We've built a strong Management team and continue to strengthen our highly qualified Board, and we'll add key people with specific expertise to our organization to drive our continued progress.

As you've just heard from Andy, we've had a productive quarter strengthening our balance sheet.

In closing, we sincerely appreciate the support of our investors. We will continue to build on what we have accomplished this year and, looking forward, we expect to have a productive and exciting 2020.

We will now open the call to your questions. Operator.

Operator:

Thank you.

If you would like to ask a question, please signal by pressing star, one on your telephone keypad. If you are using a speakerphone, please make sure your mute function is turned off to allow your signal to reach our equipment. Again, that's star, one to ask a question.

We'll now take our first question from Wangzhi Li from Ladenburg. Please go ahead, your line is open.

Wangzhi Li:

Hi, thanks for taking my questions. Congratulations in the quarter for the progress. Maybe the first question is, can you shed more color on your preparation for the Phase 1 trial, OTC, is on track for IND in your first quarter?

Joseph Payne:

Sure, Wangzhi, thanks for calling in, it's good to hear from you. We have—with respect to additional color on the OTC program, maybe I can share a little bit about the application process. We've begun drafting the IND application document itself; we've successfully completed multiple GMP-manufactured batches of the drug substance, and they've all passed their release criteria. Then with respect to the in life phase of the preclinical safety studies, that's all been completed and these of course support first in human clinical trials.

That's where we are.

Any additional questions?

Wangzhi Li:

How about the trial protocol and sites? Is that already starting, you have developed a protocol and have you selected the investigators? Any color on those fronts?

Joseph Payne:

Sure, I'll direct that question to Pad.

Padmanabh Chivukula:

Thanks, Joe. Wangzhi, hi. We will of course talk to several trial sites, and there's significant interest amongst the treating clinicians. The protocol has been reviewed by several potential investigators. In addition, Joe mentioned, we're very busy writing the IND section. Everything seem to be just on track for us.

Wangzhi Li:

Got it. Then (inaudible) to the new technology, the Starr. I kind of remember you used to had a collaboration with Synthetic Genomics, right, I think also, if I remember correctly, self-replicating? Can you provide some color on what (inaudible) technology or—any color on that?

Joseph Payne:

Our first comment is our—we are free to enable any self-replicating RNA technology. That includes Replicon technology from Synthetic Genomics and our internal Starr franchise, and so our own internal self-replicating RNA technology. We have monetized the SGI collaboration through sublicense revenue, and we've disclosed that previously. But with respect to going forward, we can independently operate with our own self-replicating RNA technology.

Wangzhi Li:

Got it. Thanks a lot.

Operator:

Thank you. We'll now take our next question from Ed Arce from H. C. Wainwright. Please go ahead.

Ed Arce:

Hi, Joe, Pad and Andy, thank you for taking my questions, and congrats on another productive quarter.

Joseph Payne:

Thanks, Ed.

Ed Arce:

I had a few questions. First is a big picture on the Lunar technology itself. I know that you've mentioned on several occasions in the past you felt that your technology was especially leading in the space, in terms of intravenous or systemic delivery, specifically to the hepatocytes, and more specifically when it's dosed chronically, or multi-dosed. I'm wondering, aside from Arcturus and your Lunar program, perhaps you could lay out what other companies in this space could have that sort of technology that really could pursue multi-dosed intravenous targeted therapy. Thanks, and I have a couple of follow-ups.

Joseph Payne:

Sure, hey, I appreciate the question, Ed. There's a small number of companies that are pursuing intravenous messenger RNA programs that we can watch out for in 2020. The first, of course, is us, Arcturus. We're pursuing the first urea cycle disorder, called ornithine transcarbamylase deficiency. This is a lipid-mediated messenger RNA therapeutic; it's a platform. Then the second company is Ultragenyx; they are pursuing glycogen storage disease type 3, and they're our partner, and they're going after—it's a liver disease and also hepatocyte program. Then the third company is Moderna, and they're initially going after organic acidemias, which is also a liver disease, and their most advanced programs are MMA and PA.

These three companies are hoping to gain some exciting progress in 2020 in the clinic for intravenous messenger RNA therapeutics, where we think there is a considerable white space commercial opportunity to fill in.

Was that helpful?

Ed Arce:

Yes. Yes, that's helpful, thank you.

Then perhaps a follow-up on the technology, and then one on the Starr, you announced this morning. You've mentioned before that there are types of—different ways of synthesizing messenger RNA molecules; one type that's a modified approach where you seek to minimize the immunomodulatory effect that has been seen in older generation compounds. Your synthesis process works well for either modified or unmodified molecules. I wanted to know if you could help provide us a little more details around why that is and how that's important, especially competitively. Thank you.

Joseph Payne:

Yes. Sure. Well, the first point here is, Arcturus utilizes a modified messenger RNA drug substance approach. We believe that that is the best approach. The second point is, Arcturus's proprietary mRNA manufacturing process works very well with our modified mRNAs, and by very well I mean increased or high purities and high yields in a timely manner.

Ed Arce:

Okay. Then …

Joseph Payne:

Is there any other …

Ed Arce:

Yes. One last one if I may. On the Starr technology you announced this morning, could you just help us understand the types of medicinal applications that you hope to pursue with this type of technology, and does it address different opportunities that aren't available with messenger RNA, or is there a significant overlap? Thanks again.

Joseph Payne:

Hey Pad, why don't you take that?

Padmanabh Chivukula:

Sure, Joe. Hi Ed, this is Pad. Yes, I mean, we believe definitely the Starr technology has certain advantages for infectious diseases and vaccine applications, and that's our initial focus. But we think there's broader applicability of this technology to other human diseases. But that data will be collected in the future.

At least initially for some of the indications that we're thinking around oncology as well as infectious disease applications, we think the technology could be very effective.

Joseph Payne:

With respect to the second part of your question, Ed, I think it's important to understand that these self-replicating RNA technologies tend to last longer than traditional mRNA therapeutics, and the Lunar delivery technology is more biodegradable. We're hoping that that translates into improved human data, being softer and less inflammatory for injection site reactions, et cetera.

Ed Arce:

Got it, that's helpful. Congrats again on the collaboration.

Joseph Payne:

All right, thank you, Ed.

Operator:

Thank you. Again, as a reminder, if you would like to ask a question, please signal by pressing star, one on your telephone keypad.

We'll now take our next question from Kumar Raja from Brookline Capital Markets. Please go ahead, your line is now open.

Kumar Raja:

Okay, thank you for taking my questions, and also congratulations on all the progress.

Maybe first on the OTC program, obviously one of your competitors had a setback (inaudible) they are discontinuing their OTC program. How does this impact your strategy in terms of the clinical development, and what kind of challenges do you foresee advancing 810 into the clinic, and maybe one more on the Starr technology.

The Starr technology in combination with the Lunar, what data do you guys have in terms of what specific cells you are able to target with this technology, and also the length of RNA that could be replicated, and also your expectation in terms of error rate with this technology. Thank you.

Joseph Payne:

Sure, hey Pad, why don't you take first crack?

Padmanabh Chivukula:

Sure. First part of your question, in rare diseases such as OTCD, it obviously works to our advantage, because clinical trial sites and patients that would otherwise have been tied up on other programs, our study's now potentially free to participate in our study. That's advantage.

In terms of strategy, it doesn't have a major impact for us. Our goal remains the same, to find the most expeditious way possible of getting our drug to OTC patients.

In terms of some of the major challenges, really to find—related to rare diseases is of course finding patients for our clinical trials. That still remains a challenge, but we plan to address that as well.

Joseph Payne:

I think the also—the other impact that the discontinuation of T Bio's program has had is more people have been bringing forward questions about biodegradability and clearance of our delivery system, which is something we showcase now and bring that forward in investor discussions. It's something we consider a significant advantage of our technology.

Padmanabh Chivukula:

In terms of your second question of the Starr technology, we obviously are currently laser-focused on advancing our OTC program to the clinic, and we're not planning to add any Starr programs to our pipeline in the near term, but we are collecting data in various preclinical models to show the superiority of this in the two applications that I mentioned, which is infectious disease and in oncology.

Joseph Payne:

From a platform business development perspective, of course our Starr technology platform is available for those strategic discussions.

Kumar Raja:

Maybe last question in terms of obviously you guys are focusing on OTC and CF. When can we expect any updates with—obviously Ultragenyx is (inaudible) advancing one of your candidates. In terms of (inaudible) programs, when can we expect in terms of any update regarding the next program that you will be moving forward into clinic?

Joseph Payne:

Yes, I'd like to refer people to the Ultragenyx website and to their investor relations and PR communications, but we're definitely supporting and doing what we can to support Ultragenyx's glycogen storage disease type 3 program, and I know they've consistently messaged an IND for 2020. That remains consistent.

Kumar Raja:

Okay, great. Thank you.

Joseph Payne:

Thank you, Kumar.

Operator:

Thank you. Again, if you would like to ask a question, it's star, one.

There are no further questions in the queue at this time. I'd like to hand the call back to Joseph Payne for any closing remarks.

Joseph Payne:

Oh, thank you.

I have no closing remarks at this time. I think we just want to appreciate, again, the support of our investors, and we look forward to reconnecting at a later time, and until then, goodbye.

Operator:

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