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Arcturus Therapeutics

Second Quarter 2019 Earnings Call

August 15, 2019

CORPORATE PARTICIPANTS

Neda Safarzadeh, D*irector, Head of Investor Relations*

Joseph E. Payne, *President and Chief Executive Officer*

Andrew Sassine, *Chief Financial Officer*

Padmanabh Chivukula, Ph.D., *Chief Scientific Officer*

CONFERENCE CALL PARTICIPANTS

Ed Arce, *H. C. Wainwright*

Kumar Raja, *Brookline Capital*

Wangzhi Li, *Ladenburg Thalmann*

PRESENTATION

Operator:

Greetings, and welcome to the Arcturus Therapeutics Holdings Second Quarter 2019 Earning Results Conference Call. At this time, all participants are in a listen-only mode. A question-and-answer session will follow the formal presentation. If anyone should require Operator assistance during the conference, please press star, zero on your telephone keypad. Please note, this conference is being recorded.

I would now like to turn the conference over to your host, Neda Safarzadeh, head of Investor Relations. Thank you. You may begin.

Neda Safarzadeh:

Thank you, Operator, and good afternoon, everyone. Thank you for joining Arcturus's earnings conference call. We are excited by this opportunity to discuss Arcturus's second quarter 2019 operating results. We are joined today by Joseph Payne, our President and Chief Executive Officer, and Andy Sassine, our Chief Financial Officer. Doctor Pad Chivukula, our Chief Scientific and Chief Operating Officer, 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's market opportunity and strategic road map. He will then review the Company's most recent partnership announcements and review important milestones for our clinical development activity. Next, Andy will be discussing the second 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 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 a statement of historical fact included in this communication regarding strategy, future operations, the status of preclinical and clinical development programs, the potential success 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 a product candidate, unexpected clinical results, and general market conditions that may prevent such achievement or performance.

Such statements are based on Management's current expectations and involve risks and uncertainties including those disclosed 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 18, 2019, and Arcturus's quarterly reports on Form 10-Q filed with the SEC on August 14, 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 any new information, future circumstances, or otherwise.

I will now turn the call over to Joe.

Joseph E. Payne:

Thank you, Neda.

Good afternoon, and thank you for joining us for our Second Quarter Results Conference Call. This is Arcturus's first ever quarterly earnings conference call, and we are very pleased for this opportunity to tell you our story about our mission to develop life-saving therapies for patients, and to provide an update on our programs.

We have had a productive few months raising capital to fund our operations. After redomiciling the Company in the U.S., we expanded and amended our present collaborations, resulting in substantial moneys being received, including $30 million from Ultragenyx in June and $4 million from CureVac in July. We received $3.3 million from Synthetic Genomics, and the CF Foundation has increased its commitment to $15 million. On top of this, we recently broadened our investor base, bringing in approximately $13 million from institutional offerings. Andy, of course, will provide a more detailed financial review later in the call.

We are, indeed, seeing progress with messenger RNA therapeutics. Messenger RNA molecules create and build life. That is what they do. Getting a messenger RNA molecule to where it needs to be, safely and effectively, will not only be a significant scientific achievement in medicine but it will also have a transformational impact on the pharmaceutical industry. At Arcturus, building and creating life-changing proteins inside a human being is our primary objective. We believe the Lunar technology is a platform delivery system that will allow us to go after targets in multiple areas, including the liver, lung, ophthalmology, infectious disease, and vaccines.

We continue to make progress on our proprietary pipeline, with our first IND anticipated in Q1 2020. That is just around the corner. We are aiming to file a second IND in late 2020, and when we include our partner program with Ultragenyx, there is the potential for a total of three INDs based on Arcturus technology to be filed in 2020.

Now let me talk about Lunar OTC, which is our most advanced clinical candidate from our wholly-owned clinical pipeline. It's also known as ARCT810, which will be the subject of our first IND application.

Now we are developing ’810 to treat ornithine transcarbamylase deficiency, which we also refer to as OTC deficiency. OTCD is the most common urea cycle disorder, where you have neurotoxic ammonia being converted to water-soluble urea that can be excreted in the urine. The deficiency in OTC causes elevated blood ammonia, which can lead to neurological damage, coma, and death. The present standard of care involves a strict diet of low protein and high fluid intake, plus ammonia scavengers like sodium phenylbutyrate. It does not effectively present spikes of ammonia, however. Sadly, many OTC deficiency patients are typically referred for liver transplants.

Now, our Lunar OTC program aims to restore the enzyme function in the liver, with the potential to restore normal urea cycle activity, detoxify the ammonia, prevent the neurological damage, and remove the need for liver transplantation. Now, we believe there is ten thousand worldwide OTC patients, which represents a $500 million potential annual sales market.

Importantly, Arcturus recently received orphan drug designation from the U.S. FDA in late June.

We have completed the manufacturing of drug substance and drug product, using our proprietary processes. We have now completed multiple batches of Lunar-formulated OTC mRNA drug product at 10 grams each. We believe the current inventory is sufficient to support IND-enabling studies and early clinical development.

We have also initiated GLP tox studies in two species, that we expect to complete before year end.

In parallel, we have scheduled to manufacture the first batches of GMP drug product that we can use in clinical trials.

Moving on to our CF program. We were very excited to announce on August 1 the Cystic Fibrosis Foundation has increased its commitments to $15 million, and that was in conjunction with an amended agreement to advance Lunar CF, which is a novel messenger RNA therapeutic formulated with Arcturus’s Lunar delivery technology. The goal of this multi-year program is to create mRNA therapies to treat people with cystic fibrosis. We want to develop methods to deliver RNA into the cells of the lung, and file an IND application for a therapeutic candidate. This was a collaboration that began in 2016. With this expanded collaboration, we are now sufficiently funded to advance the Lunar CF program to IND submission. We expect a development candidate nomination in the first quarter of 2020, and together with the CF Foundation, we can then expect to file an IND in late 2020.

As a reminder, cystic fibrosis has a worldwide prevalence of about 70,000 patients. CF is caused by genetic mutations in the CFTR gene, that cause thick mucus build-up in lung airways. A messenger RNA replacement therapy has the potential to deliver a new, fresh copy of the transporter CFTR into these lungs of CF patients, independent of any genotype. We estimate the class one CF market potential is $900 million of annual sales, and we look forward to providing future updates as this program advances.

Arcturus and Ultragenyx originally signed a license agreement in 2015 to develop messenger RNA therapeutic candidates for certain rare disease targets. This past June, we announced an expanded collaboration to discover and develop messenger RNA, DNA and siRNA therapeutics for up to 12 rare disease targets. Under the terms of the expanded agreement, Ultragenyx paid $6 million in cash to Arcturus and purchased 2.1 million shares of common stock at $10 per share. Ultragenyx is now Arcturus's largest shareholder and has a seat on the Company's Board, in addition to an observer role.

The first disclosed indication under the collaboration is glycogen storage disease type 3. An IND application for this mRNA therapeutic program, also known as UX053, is expected to be filed in 2020. We are indeed

excited to expand our partnership with Ultragenyx as we work together to find transformative solutions for rare diseases.

I want to take a moment now to emphasize some data that Arcturus recently presented on our Lunar technology, and more specifically on the biochemical properties of our lipids. We believe these data are very very relevant, as they reflect well on the strong safety profile of our technology, as well as on our competitive positioning. The data that we presented demonstrates that the key Arcturus lipid that makes up the major portion of the Lunar technology is quickly biodegraded with a half-life of 20 hours. We have shown that we can safely administer eight weekly doses of Lunar-formulated non-coding RNA at 3 mgs per kilogram, and we can do this in primates for a total of 24 milligrams per kilogram over two months. To put this in perspective, the FDA-approved dose for what is considered to be the existing clinical gold standard for lipid nanoparticle RNA delivery is only 0.3 mgs per kilogram, once every three weeks dosing.

As we think about the second half of 2019, the remainder of this year, we plan to participate in numerous scientific conferences, including the OTS or the Oligonucleotide Therapeutics Society conference in October, and also the International mRNA Health Conference that's held in Berlin. We will provide you with an update when we have more details there.

We have signed multiple partnership agreements that both validate our technology and provide financial backing to fund our research. The aggregate of these agreements contains more than $1 billion in potential milestones and royalties to Arcturus. We've been able to build upon track records in 2019, and expanding our collaborations with both the CF Foundation and with Ultragenyx, resulting in additional payments to Arcturus as well as an increase in committed resources for the programs covered.

Our business development activities toward growing our platform continue to be a high priority. We have several ongoing evaluations with potential partners and continue to collect new and promising data as we consider licensing our technologies, especially our Lunar technology, to enable large nucleic acid medicines. This includes replicon RNA, gene editing RNA, next generation DNA therapeutics. We will, of course, provide you updates as these business development activities mature.

I will now turn the call over to Andy Sassine for a financial review. Andy.

Andrew Sassine:

Thank you, Joe, and good afternoon, everyone. We issued a press release earlier today that included a financial update on the second quarter ended June 30, 2019, which I will briefly summarize.

Collaboration revenue was $10.2 million during the quarter ended June 30, 2019, compared to $2.4 million in the comparable period in 2018. Approximately $7 million is nonrecurring revenue from three one-time events.

First, we recognized $3.3 million from our Synthetic Genomics agreement related to sublicense revenues from multiple parties.

Second, we recognized $3 million from our partner CureVac in connection with the Termination Agreement related to the OTC program.

Finally, we recognized approximately $1 million from Ultragenyx agreement as nonrecurring revenues.

Operating expenses were $10.7 million in the quarter ended June 30, 2019, compared to $12.5 million in the quarter ended June 30, 2018. The June 2018 quarter included approximately $5 million in expenses related to the proxy event last year.

Net loss for the quarter ended June 30, 2019 was approximately $0.7 million, or $0.07 per basic and diluted weighted average shares outstanding, compared with a net loss of $10 million or $0.99 per share in the comparable period in 2018.

At June 30, 2019, Arcturus had cash and cash equivalents totaling $55.8 million, compared to $36.7 million at December 31, 2018. The majority of the increase was due to the expanded collaboration agreement we announced with Ultragenyx Pharmaceuticals in June. We received $30 million from Ultragenyx, comprised of a $24 million equity investment at $10 per share and a $6 million upfront payment.

Subsequent to the end of the fiscal quarter, we announced three significant transactions which further improved our liquidity.

First, we received a $4 million payment from CureVac at the end of July in connection with the Termination Agreement for the co-development of ARCT810 as a therapy for OTC deficiency.

Second, we announced two registered direct offerings of our common stock at $11.50 per share to certain institutional investors that, in the aggregate, raised net proceeds of $12.2 million. The two offerings were for an aggregate of 1,145,653 shares of common stock. This was the first institutional offering completed by Arcturus, and we are grateful for the strong support from our existing and new shareholders.

Finally, the Cystic Fibrosis Foundation increased their commitment up to $15 million to fund a Lunar-CF program through IND. We anticipate receiving the first payment of $4 million by the end of August.

In total, we added an additional $20 million in cash since the end of the quarter, and we believe our cash resources should be sufficient to support our operations through the end of fiscal year 2020.

If we take into account the three aforementioned transactions, our common shares outstanding would be 14.3 million shares, approximately.

For further details on our financials, including our results for the six-month period ended June 30, 2019, please refer to our most recent Form 10-Q filed with the SEC yesterday.

I will now turn the call back to Joe to wrap up our presentation and answer questions from analysts.

Joseph E. Payne:

Great. Thanks, Andy, and Pad and Neda, and thank you to all, and all the broader team at Arcturus. This is a very exciting time for Arcturus. We believe that the progress we are making on our pipeline, combined with the success of our business development and financial activities, will serve to reinforce our position as a leader in RNA medicines. We are energized by all the activity, as you can understand, and all the progress happening around the Company and our delivery platform, and we look forward to a productive second half of the year and further meaningful news flow in 2020.

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

Operator:

Thank you. At this time, we will be conducting a question-and-answer session. If you would like to ask a question, please press star, one on your telephone keypad. A confirmation tone will indicate your line is in the question queue. You may press star, two if you would like to remove your question from the queue. For

participants using speaker equipment, it may be necessary to pick up your handset before pressing the star key.

Our first question comes from the line of Ed Arce with H.C. Wainwright. Please proceed with your question.

Ed Arce:

Hi, Joe and team. Congrats on all the recent progress, especially the new collaborations.

Couple questions for me. First, I would like if you could review again the data, in particular to the delivery of your proprietary lipids; I think that is quite key to the overall platform. If you could review and perhaps expand upon that a bit, especially relative to not only the standard of care you mentioned, but just the broader space. Thanks.

Joseph E. Payne:

Oh. Hi, Ed. Thanks for the question. Just to reiterate, it sounds like you want some more color on the advancements we've made with respect to data pertaining to our delivery. I'll give the first crack of that, and Pad, feel free to chime in.

But, I think, first and foremost, we recently shared the biodegradability of our lipid in our Lunar technology. This is, of course, very important because accumulation of lipids, after multiple dosing, can be a significant concern with respect to toxicology. It's ideal to have a lipid-mediated delivery system that does its job and then degrades rapidly, and prevents this accumulation from occurring. We've reported a 20-hour half-life.

To put some color on that, that is not 20 days. You can imagine, if you're trying to design a therapeutic that's being dosed weekly or biweekly, that it would be a significant concern if half your lipid is still around after three weeks, right? Just to confirm that our lipid half-life that we've evaluated and shared that data recently is 20 hours. That just gives us more flexibility with respect to dosing and the timing of dosing.

Other than that, Pad, is there any other additional …?

Padmanabh Chivukula:

No. Thanks, Joe. The only other thing I would add is that we've provided data in our deck previously that we do the—our therapeutic for OTC is delivered by IV administration, and we get substantial uptake into the liver hepatocytes, both in rodent species and in non-human primates.

Joseph E. Payne:

Right. This uptake includes the periportal portion of the liver, where we feel it's a very important portion of the liver that's responsible for the urea cycle disorders, including ornithine transcarbamylase deficiency.

Ed Arce:

Great. That's helpful.

The other question I had, I guess, is around your lead program, the OTC that you have now for a first quarter IND, first quarter next year. Wondering if you could just go over what's left to be completed between now and then, what specifically are you working on, the progress of that, and if there's any sort of gating factors to the work that's left to be completed?

Joseph E. Payne:

Great question, Ed. Pad, why don't you provide some color there?

Padmanabh Chivukula:

Thanks, Joe. Yes. Our team is diligently working with outside medical writers and vendors and various regulatory consultants to efficiently and strategically assemble our first IND filing. We made, obviously, some key hires and brought on experienced folks over the last quarter, putting us in great position as we approach our first in-human studies with our Lunar program. We actually look forward to reporting our success and filing the IND in the first quarter of 2020.

Joseph E. Payne:

Then with respect to—I did highlight on the call the status of our GLP tox studies and our drug substance and drug product manufacturing efforts and timeline there as well.

Ed Arce:

Yes. That's great. I'll jump back in the queue. Thanks, again.

Operator:

Once again, if you would like to ask a question, please press star, one on your telephone keypad. Once again, if you would like to ask a question, please press star, one on your telephone keypad.

Our next question from someone of Kumar Raja with Brookline Capital. Please proceed with your question.

Kumar Raja:

Hi, thanks for taking my questions, and also congratulations on all the progress.

Maybe on ARCT810, obviously one of your competitors, Translate, they had a setback earlier this year, they had a clinical hold. Then it was lifted, but they are allowed to do a single ascending dose. What I'm trying to get a sense here is like, what are you guys doing in terms of preclinical studies so that you are able to move very smoothly in the clinic, as well as able to dose-escalate?

Padmanabh Chivukula:

Hi Kumar. This is Pad, and I'll answer that question. Obviously, we don't know the specifics related to the clinical hold for Translate. What we understand is there were both clinical and non-clinical issues. But as for ARCT810, well, we're not prepared to discuss our non-clinical strategy currently, but we are conducting a very thorough characterization of the safety profile with non-clinical studies that support our clinical study design for our first in-human, for the Lunar platform in OTC patients. We had feedback from the FDA in regards to our non-clinical plan, and we've incorporated that into our studies. We believe our non-clinical and clinical approaches for ARCT810 first in-human should put us in great position to conduct our (inaudible) portions into the clinic without any incident.

Kumar Raja:

Okay. In terms of the Lunar-CF, obviously, in the CF patients, we see excess of mucus there. What are your expectation in terms of nebulization as a delivery vehicle there? How effectively you think you can get a drug there?

Also, you guys are trying to do some ferret model studies; how does that reflect compared to what we see in humans?

Joseph E. Payne:

Sure. Pad, why don't you address that?

Padmanabh Chivukula:

Sure. What we've shown so far is, of course, we've collected and shared data on the stability of the Lunar formulation in the CF sputum of patients. We observed the stability of the mRNAs improved greatly with our platform. Does that—is that—have you seen some of our data that we presented?

Kumar Raja:

Yes, I have.

Padmanabh Chivukula:

Good. We believe—Because of that, we think we have a great platform technology that can address the mucus question.

Kumar Raja:

You guys had talked a little bit about the lipid degradation earlier in the call. You guys have been making some progress in ophthalmology also. I wanted to get a sense, what is the expectation in terms of lipid degradation-slash-accumulation in the eye, how it is different from liver or epithelial cells in the lungs? Also, are you—what are your expectations? Do you think you will need intravitreal injection or you think you will be able to come up with a topical formulation for ophthalmology indications?

Joseph E. Payne:

Great, thanks, Kumar. Lunar delivery to the eye, at least our proof of concept studies that we've completed internally, has been achieved with intravitreal injections, so not topical or intravenous injections.

With respect to the degradation data of the lipid technology, we have collected and presented lipid degradation data for intravenously-dosed Lunar formulations, and we reported that 20-hour half-life or in vivo half-life for the ATX lipid. But we have not shared our lipid degradation data for inhaled or intravitreal injected Lunar formulations, although we are encouraged by the intravenous data that we've shared, and we believe that we have not only designed our lipids from the very onset to be biodegradable.

Kumar Raja:

Okay, great. Congratulations once again. Thank you.

Joseph E. Payne:

Thanks, Kumar.

Operator:

Our next question comes from the line of Wangzhi Li with Ladenburg Thalmann. Please proceed with your question.

Wangzhi Li:

Hi. Thanks for taking my question and also congratulations on the quarter.

I want to follow up on the lipid periods question because that's the key differentiation of your Lunar platform on the other lipid nanoparticle technologies. As you mentioned the half-life of 20 hours, maybe give me more context, what—is there FDA guidance or preference in terms of what kind of timeframe of lipid period or half-life is optimal or desired, in context of Translate Bio's IND hold for this kind of use?

Joseph E. Payne:

Sure. Sure. I think it'll help, first of all, to clarify or help people understand the half-life of the OTC itself. Ornithine transcarbamylase is this enzyme that resides in hepatocytes in the liver. Once you build or create and make this enzyme, it lasts a long time. It has a half-life of over 10 days, approximately 12 days. Just theoretically, I think it's helpful to understand that your lipid delivery technology needs to remove and degrade and clear from the system before the next injection, but because the ornithine transcarbamylase enzyme itself lasts for so long, that it's attractive theoretically to see that our half-life for the lipid delivery technology is only 20 hours.

Then to put some additional color, there's other lipids like MC3 that have data as well, that you can look to as a standard to compare to. That's injected once every three weeks.

Okay?

Wangzhi Li:

Got it. Maybe maturity by in terms of the OTC program Phase 1, any color on the trial design at the moment, in terms of dosage frequency or how many doses you're going to try?

Joseph E. Payne:

With respect to the clinical trial design? Pad, why don't you answer that?

Padmanabh Chivukula:

Sure. Wangzhi, this is Pad. Yes, I think it's too early to give you that sort of granularity around our clinical trial design. As you know, we're rapidly progressing in completing our GLP tox studies, and we'll be filing the IND soon. But it is definitely premature to provide that sort of granularity, but we will be updating the market soon about that.

Joseph E. Payne:

We have met with multiple regulatory agencies on both continents, and received their guidance and feedback with respect to some aspects of our clinical plan. We do plan to go directly into patients. But as for additional details pertaining to that clinical plan, those haven't been disclosed, as Pad mentioned. We can provide more detail at a later time.

Wangzhi Li:

Okay got it, thanks. Then also shifting gears to the Lunar-CF program, it's great the CF Foundation committed to $15 million R&D. Maybe, if you can, provide more color on what really triggers that commitment, and what remains to be done before you file IND late next year?

Joseph E. Payne:

Sure. We have messaged that we plan to nominate a development candidate again in the first quarter of 2020 and an IND at year end of 2020. As for the timing of other aspects of that program, we haven't disclosed those details, but I assume that as the program ages we will be sure to update the markets accordingly.

Wangzhi Li:

What are triggers? I know you started the collaboration a while ago, so I guess what really triggers these commitments from the foundation for $15 million investment?

Padmanabh Chivukula:

I think that obviously this collaboration has been going on for a few years, and they're impressed by the progress that we're making in the first half of the grant, and they want to—and the data they've seen, and because of that they want to support us to develop this program into the clinic.

Joseph E. Payne:

The most interesting or impressive data that we've collected is functional delivery of several different types of messenger RNA molecules to bronchial epithelial cells. This means that we're not only—our delivery technology is not only getting to these cells, but getting inside of them and breaking out of the endosome and delivering the messenger RNA itself, in mice.

Wangzhi Li:

Got it. The last question is, for the CF program, you're going to use a nebulizer, right?

Joseph E. Payne:

That is correct. Yes. We plan to—now, with respect to which specific inhalation medical device, we have not disclosed that yet. At some point—but we have not disclosed which one we're going to proceed with yet.

Wangzhi Li:

All right. They are all my questions. Thanks, again.

Joseph E. Payne:

Sure, thanks, Wangzhi.

Operator:

Ladies and gentlemen, we have reached the end of our question-and-answer session, and I would like to turn the call back over to Mr. Joe Payne for any closing remarks.

Joseph E. Payne:

Okay, it sounds like that's all the time we have today. Thank you, all, for participating in Arcturus's first quarterly conference. We appreciate the time you've taken to listen, and we look forward to our next call. Bye for now.

Operator:

This concludes today's teleconference, you may now disconnect your lines at this time, thank you for your participation, and have a wonderful day.