ARROWHEAD PHARMACEUTICALS

Fiscal 2017 Third Quarter Conference Call – Prepared Remarks

August 3, 2017

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Pharmaceuticals conference call. Throughout today's recorded presentation all participants will be in a listen-only mode. After the presentation, there will be an opportunity to ask questions. I will now hand the conference call over to Vincent Anzalone, Vice President of Investor Relations for Arrowhead. Please go ahead Vince.

Vince Anzalone

Good afternoon everyone. Thank you for joining us today to discuss Arrowhead's results for its fiscal 2017 third quarter ended June 30, 2017. With us today from management are president and CEO Dr. Christopher Anzalone, who will provide an overview and Ken Myszkowski, our chief financial officer, who will give a review of the financials. Dr. Bruce Given, our chief operating officer and head of R&D, will also be available during the Q&A portion of the call.

Before we begin, I would like to remind you that comments made during today's call contain certain forward-looking statements within the meaning of Section 27(A) of the Securities Act of 1933 and Section 21(E) of the Securities Exchange Act of 1934. All statements other than statements of historical fact, including without limitation those with respect to Arrowhead's goals, plans, and strategies are

forward-looking statements. These include statements regarding our expectations around the development, safety and efficacy of our drug candidates, projected cash runway, and expected future development activities. These statements represent management's current expectations and are inherently uncertain. Thus, actual results may differ materially. Arrowhead disclaims any intent and undertakes no duty to update any of the forward-looking statements discussed on today's call.

You should refer to the discussions under risk factors in Arrowhead's annual report on Form 10-K and the Company's subsequent quarterly reports on Form 10-Q for additional matters to be considered in this regard, including risks and other considerations that could cause actual results to vary from the presently expected results expressed in today's call.

With that said, I'd like to turn the call over to Dr. Christopher Anzalone, President and CEO of the Company. Chris?

Chris Anzalone

Thanks, Vince. Good afternoon everyone and thank you for joining us today.

Over the last quarter we have focused on meeting the aggressive timelines we set internally for our development programs, while continuing with disciplined cash management. We are excited about our new platforms, our lead drug candidates are progressing rapidly toward the clinic, and our partnership with Amgen continues to be highly productive. These are all important steps for us as a company and give us confidence to push forward with our new and diverse pipeline of RNAi therapeutics.

Since we made the decision in November of 2016 to discontinue development of prior generation drugs, ARC-520, ARC-521, and ARC-AAT, that utilized the EX1 delivery vehicle, we have been quiet about data on our new platform and the drug candidates being built upon it. Our plan has been to hold an analyst R&D day to provide a comprehensive update about the technology and our lead programs. We think the most effective time to hold this event is when our data across multiple programs have sufficiently matured and when we are in reasonable proximity to filing with regulators to start first in human studies. We are just about there.

The analyst R&D day is scheduled for Thursday, September 14th in New York City. It will be held live so analysts and institutional investors can attend in person, and there will also be a webcast so those not in attendance can listen in. That's just 42 days from now, so for today's call I want to talk about the event and what to expect from the presentations.

There will be 2 primary components: First, we will discuss our new base platform; then, together with expert KOLs, we will discuss the three most advanced programs in our pipeline and the clinical and treatment dynamics of the disease states those three programs will be addressing.

Regarding the platform presentation, we will go over the general design and benefits of our new delivery platform that we call TRMs™, or Targeted RNA Molecules. Importantly, TRMs™ are proving capable of efficient delivery and knockdown of target genes not just in the liver, but also *in tissues outside the liver* without the need for an engineered endosomal escape component, as we had in our prior generation technology. We plan on showing some example data demonstrating the activity of 3 different TRMs each optimized for different uses:

1) A liver targeted TRM that we are using for several drug candidates;

2) A tumor targeted TRM used in our ARO-HIF2 candidate, delivered systemically; and

3) A TRM targeted to a non-liver, non-tumor tissue that we will disclose at the event.

We have always believed that for RNAi to achieve its full potential as an important new therapeutic modality, it must address diseases beyond the liver. Arrowhead is leading this expansion with TRMs targetable to new organ systems, and this has matured from goal to reality. We think this represents a giant leap forward for the field and, we believe, presents a host of new opportunities unique to Arrowhead.

We will discuss the unifying characteristics and strategies behind TRMs that maximize target gene knockdown and minimize off-target exposure. We see TRMs as a powerful, enabling solution for RNAi therapeutics against a wider universe of potential diseases that were previously not accessible with other approaches.

During the next component of the analyst day, we will go through a pipeline update that includes data on three of our lead programs. This will include guidance on when we expect to file with regulators to begin first-in-human studies. Those lead programs are:

- ARO-AAT, which is a second generation therapeutic against liver disease associated with alpha-1 antitrypsin deficiency.
- ARO-HBV, which is a third generation therapeutic against chronic hepatitis B virus infection.

- ARO-APOC3, our previously undisclosed candidate designed to knockdown production of apolipoprotein C-III, or ApoC3, to reduce elevated plasma triglyceride levels, which is an independent risk factor for cardiovascular diseases.

We have officially nominated the ARO-HBV and AAT candidates and final IND-enabling steps, including manufacturing and GLP toxicology studies, are scheduled to begin shortly.

Each update will include preclinical data, discussion of the unmet need and opportunity for a new or improved therapy, and for ARO-AAT and ARO-HBV, a recap of some of the important things we learned in our prior development programs that are helping to inform the development path of these new product candidates. We think the insights we have about HBV and alpha-1 liver disease represent real competitive and strategic advantages and enable us to move with speed and precision once the clinical programs begin.

The final component of the analyst day will be discussions from key opinion leaders in each pipeline lead disease area about disease biology, epidemiology, unmet need, current therapies, and how we think our drug candidates would fit into the treatment landscape. They will each give a brief presentation and will also be available for Q&A when the presentations conclude. The KOLs currently scheduled are:

- Dr. Jeffrey Teckman from the Saint Louis University School of Medicine for alpha-1 liver disease;
- Dr. Stephen Locarnini from the Victorian Infectious Diseases Reference Laboratory for HBV; and,

- Dr. Ira Goldberg from the NYU Langone Medical Center for cardiovascular disease

The analyst day will be packed full of new information that we have been excited about for some time and we are eager to share it with you. But, that will likely just be the beginning of our efforts to get the word out about the progress our R&D organization has made and continues to make. We have substantially more data than we can present during that event, so we intend to look for opportunities to present additional data at appropriate medical meetings in the future, as we have always done in the past. In addition, as more candidates using liver TRMs, tumor TRMs, and new organ system TRMs become further advanced, we intend to give updates and guidance when possible.

With that overview, I'd like to turn the call over to Ken Myszkowski, Arrowhead's Chief Financial Officer?

Ken?

Ken Myszkowski

Thank you, Chris, and good afternoon everyone.

As we reported today, our net loss for the three months ended June 30, 2017 was $5.3 million, or $0.07 per share based on 74.8 million weighted average shares outstanding. This compares with a net loss of $19.4 million, or $0.32 per share based on 60 million weighted average shares outstanding, for the three months ended June 30, 2016.

Revenue for the three months ended June 30, 2017 was $9.4 million, compared to $40 thousand for the three months ended June 30, 2016. This increase is driven by the upfront payments received from our collaboration agreements with Amgen, and these payments will be recognized as revenue over the next several quarters.

Total operating expenses for the three months ended June 30, 2017 were $14.9 million, compared to $19.4 million for the three months ended June 30, 2016. The decrease is driven by the discontinuation of the clinical trials related to our previous clinical candidates.

Net cash used by operating activities during the three months ended June 30, 2017 was $10.4 million, compared with net cash used of $18.3 million during the three months ended June 30, 2016. Cash used in operating activities decreased as compared to last year due to lower spending related to clinical trial expenses.

Turning to our balance sheet, our cash and short-term investments combined totaled $75.1 million at June 30, 2017, compared to cash of $85.4 million at September 30, 2016. We have invested $36.8 million in short-term corporate bonds that mature within the next 12 months. Our total cash and investments balance decreased $10.2 million as compared to our September 30, 2016. Our cash burn was partially offset by the $30 million upfront payment and $12.5 million equity investment from Amgen.

Our common shares outstanding at June 30, 2017 were 74.8 million.

With that brief overview, I will now turn the call back to Chris.

Chris Anzalone

Thanks Ken.

Thank you all for joining us today and I hope everybody can listen to the presentations at the Analyst R&D Day on September 14. We think our new TRM platform, as it is today, is the product of many important breakthroughs. It was made possible through years of work and the exploration of countless different technology platforms by talented Arrowhead scientists who are focused on innovation.

We feel that Arrowhead has always been the high science RNAi company and we think that our presentations at the analyst day next month will continue to support that view. We are thrilled to share our progress and we hope that everyone will be as excited about Arrowhead's future as we are.

I would now like to open the call to your questions. Operator?

Operator

Operator opens the call to questions …