

ARROWHEAD PHARMACEUTICALS

Fiscal 2018 First Quarter Conference Call – Prepared Remarks

February 9, 2018

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 2018 first quarter ended December 31, 2017. With us today from management are president and CEO Dr. Christopher Anzalone, who will provide an overview of the quarter; Dr. Bruce Given, our chief operating officer and head of R&D; and Ken Myszkowski, our chief financial officer, who will give a review of the financials. We will then open up the call to your questions.

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.

It's been a short time since we held our last earnings call for fiscal 2017 year-end, but we have already made substantial progress towards our 2018 goals. It is gratifying to see how far we have come in the last twelve months and exciting to see what we are expecting to accomplish in 2018. We are positioned to go from 0 to 5 clinical programs in 12 months and I want to applaud the hard work of all the smart, talented, and driven folks at Arrowhead who are making this possible.

Let's begin with our primary goals for calendar 2018. They are:

1. Complete dosing our Phase 1/2 study of ARO-HBV
2. Complete dosing our Phase 1 study of ARO-AAT
3. Organize an R&D day to discuss the new pulmonary platform
4. Execute a business development collaboration
5. File a CTA for ARO-APOC3
6. File a CTA for ARO-ANG3
7. File a CTA for our first inhaled pulmonary program, ARO-Lung1; and
8. Present data at appropriate scientific conferences

All of our programs are based on our TRiMTM platform, which utilizes ligand-mediated delivery and is designed to enable multiple tissue targeting, while being structurally straight forward. It is the product of more than a decade of research at Arrowhead using targeted drug delivery vehicles for RNAi. TRiMTM technology enables Arrowhead's RNAi therapeutics to retain the maximal activity achieved with prior generation technologies, but with a more structurally straight forward molecule that offers several potential advantages. These include:

1. Simplified manufacturing and, therefore, reduced costs;
2. Multiple routes of administration, including subcutaneous injection and inhaled administration;
3. Potential for improved safety; and
4. The promise to bring RNAi to tissues outside the liver, which would represent a big leap forward for the field and a substantial competitive advantage for Arrowhead.

For a more comprehensive presentation on the TRiM™ platform, you can view the webcast recording of an R&D day that we hosted in September of 2017, which can be found on the investor section of the Arrowhead website.

One significant benefit of the TRiM™ platform is that it allows us to move very fast from an idea to an optimized clinical candidate, as demonstrated by the speed at which we were able to advance our two lead candidates. ARO-AAT is our second generation subcutaneously administered candidate for the treatment of alpha-1 antitrypsin deficiency liver disease, and ARO-HBV is our third generation subcutaneously administered clinical candidate for the treatment of chronic hepatitis B virus infection.

We went from re-starting our preclinical models and designing new triggers at the end of 2016 to selecting final candidates, manufacturing the drug supply, completing GLP tox studies, and filing CTAs just 12 months later. The ARO-AAT filing was a quarter ahead of our guidance and the ARO-HBV filing was two quarters ahead of guidance. This was the result of monumental effort by our R&D organization, but also related to our technology and corporate culture. The TRiM platform allows us to start and advance new product development programs with extreme precision, and we are relentless in our pursuit of speed. We look for every opportunity to shave days, weeks, and sometimes even months off the development timelines.

Since filing our CTAs, we have had positive interactions with the review committees for both the ARO-AAT and ARO-HBV programs, and only a few questions have been asked. Drug will be available at the sites in March and we believe we are on track to begin dosing this quarter, although, of course, this will require final regulatory and IRB approval.

For both candidates we intend to use fixed dose levels as opposed to dosing on a milligram per kilogram basis. We hope this will simplify the process for the pharmacists at the sites and reduce the risk of dosage error. It ultimately may also give opportunities for simplified commercial dosage forms such as pre-filled syringes or other options that make it easier for patients and physicians to administer the products.

We have a good amount of experience with these diseases from our prior clinical programs that spanned 17 countries and included more than 300 human study subjects receiving over 800 dose administrations. Our relationships with influential investigators throughout the world remain strong. This is partly due to the high quality science that underlies our candidates and our proven ability to effectively manage complex clinical studies.

For both candidates, the first-in-human studies will evaluate safety, tolerability, pharmacokinetic, and pharmacodynamic effects. For ARO-AAT the pharmacodynamic measure will be the reduction in serum alpha-1 antitrypsin levels. For ARO-HBV, we intend to assess all measurable viral markers, including s-antigen, DNA, RNA, e-antigen, and core-related antigen in chronic HBV patients.

Let's now turn to other programs in our pipeline.

During our R&D day last year, we talked about our expanded cardio-metabolic pipeline, which now includes ARO-APOC3 and ARO-ANG3.

ARO-APOC3 is designed to reduce production of Apolipoprotein C-III, or apoC-III, which is a component of triglyceride rich lipoproteins, including VLDL and chylomicrons, and is a key regulator of triglyceride metabolism. Elevated triglyceride levels is an independent risk factor for cardiovascular disease, and severely elevated triglycerides in patients with familial chylomicronemia syndrome, or FCS, can result in acute and potentially fatal pancreatitis.

ARO-ANG3 is designed to reduce production of angiopoietin-like protein 3, or ANGPTL3, a liver synthesized inhibitor of lipoprotein lipase and endothelial lipase. ANGPTL3 inhibition has been shown to lower serum LDL, serum and liver triglycerides, and has genetic validation as a novel target for cardiovascular disease.

Both APOC3 and ANGPTL3 appear to be well validated targets and could be of interest to large pharmaceutical companies with the resources to run large pivotal cardiovascular studies and ultimately market such drugs. We believe that ARO-APOC3 and ARO-ANG3 will be the first RNAi candidates against these targets to reach the clinic and that we will have certain advantages against other potentially competing modalities. As such, we believe we are well positioned to attract good partners for these programs when the time is right to enter into collaborations. We should be on track to file CTAs by the end of 2018 for both programs.

We are also moving forward with disease targets outside the liver. The first two of which are ARO-HIF2, which is being developed for the treatment of clear cell renal cell carcinoma, and ARO-LUNG1 against an undisclosed lung target. ARO-HIF2 is moving toward a planned CTA in 2019 and ARO-LUNG1 has a planned CTA around the end of 2018.

We have not disclosed much about our lung targeting programs. We have generated some very promising data showing that after inhaled administration we can achieve substantial knockdown of lung expressed targets with long duration of effect. We can foresee a host of new diseases we can attack and multiple targets for each of those diseases. As such, our TRiM-based pulmonary platform feels like a franchise unto itself. This could enable us to drug certain targets in ways that no other company is currently capable. Needless to say, we see substantial value creation opportunities here. Our goal is to have an R&D day around the middle of the year to discuss that program in more detail and reveal our first disease target. We are really excited about this and we will provide more information about the R&D day presentation in the coming months.

Having a reliable technology platform like TRiMTM gives us far more product development opportunities than we can currently move forward ourselves. Because of this, one of our key strategic priorities is to seek development partners for some of our programs and retain global product rights for others. As I mentioned, we see ARO-APOC3 and ARO-ANG3 as potentially attractive partner programs at some point, and we see the pulmonary platform as target-rich for collaborators as well.

In September 2016 we signed our first partnership and collaboration agreement for the TRiMTM platform with Amgen. That deal covered two cardiovascular targets. One against lipoprotein(a), or Lp little a, which is now referred to as AMG-890 by Amgen. The second, which we call ARO-AMG1, is against an undisclosed target. We are thrilled to be working with a company like Amgen and both of these programs are progressing well.

ARO-F12 is another candidate intended as a partnering opportunity. It is designed to reduce the production of factor 12, which may provide benefits in both thrombosis and hereditary angioedema. We previously had it listed on our pipeline chart as "available for partnering". We will discuss ARO-F12 with interested parties, but we do not currently have a development partner and we do not intend to initiate clinical studies on our own, so we have removed it from our pipeline. We will provide updates if this changes.

We see partnering as a way to maximize the value of our technology. It allows us to focus our internal development resources on our lead candidates and also gives us exposure to additional high value opportunities that may be beyond the reach of a small biotech company. In addition, partnering can be an important source of capital that can supplement our need to access the capital markets.

That leads me to our recent equity financing with gross proceeds of \$60.4 million. This was a very successful, oversubscribed transaction that accomplished several important goals. It strengthened our balance sheet so that we can move our pipeline to key milestones that could represent significant value catalysts. A stronger balance sheet also puts us in a better negotiating position should additional business development opportunities arise. The equity raise also gave us a much improved institutional investor base. Since we discontinued the ARC-520, ARC-521, and ARC-AAT clinical programs in 2016, we have been substantially under-owned by institutions. We still have a lot of opportunities to bring in new funds, but this financing was a good first step toward rebuilding a long-term supportive shareholder base.

With that overview, I'd now like to turn the call over to Ken Myszowski, Arrowhead's CFO, who will review our financials?

Ken?

Ken Myszkowski

Thank you Chris, and good afternoon everyone.

As we reported today, our net loss for the quarter ended December 31, 2017 was \$13.2 million, or \$0.18 per share based on 74.8 million weighted average shares outstanding. This compares with a net loss of \$12.1 million, or \$0.17 per share based on 71.4 million weighted average shares outstanding, for the quarter ended December 31, 2016.

Revenue for the quarter ended December 31, 2017 was \$3.5 million, compared to \$4.4 million for the quarter ended December 31, 2016. Revenue in each period relates to the recognition of the upfront payments received from our collaboration and license agreements with Amgen. Of the total upfront payments of \$35 million, all but \$1.9 million has been recognized as revenue to date, and the remainder is anticipated to be recognized over the next 9 months.

Total operating expenses for the quarter ended December 31, 2017 were \$17.3 million, compared to \$19.3 million for the quarter ended December 31, 2016. This decrease is primarily due to the discontinuation of our previous clinical trials in late 2016.

Net cash used by operating activities during the quarter ended December 31, 2017 was \$14.7 million, compared with net cash provided from operating activities of \$10.0 million during the quarter ended December 31, 2016. The key driver of this

change was the \$30 million upfront payment received from Amgen in 2016 associated with our ARO-LPA collaboration and license agreement.

Turning to our balance sheet, our cash and short-term investments totaled \$50.7 million at December 31, 2017, compared to \$65.6 million at September 30, 2017. The decrease in our cash was primarily driven by cash used for operating activities. In January 2018, we completed an equity financing, issuing 11.5 million shares, which resulted in \$56.8 million of net cash proceeds to the Company. This financing, along with our existing cash and short-term investments, provided us with more than \$100 million of liquid assets which will allow us to continue to advance our pipeline through the clinic for many quarters.

Our common shares outstanding at December 31, 2017, were 74.9 million.

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

Chris Anzalone

Thanks Ken.

We have great expectations for 2018 and believe that the company you will see this time next year will be substantially different than the one you see today. We believe that we are the fastest and most innovative company in the field and, because of this, we are at the door of disruptive opportunities to change medicine and public health in a variety of areas. To both new and existing investors, we think we can accomplish a lot together, and we sincerely thank you for joining us.

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

Operator

Operator opens the call to questions ...