ARROWHEAD RESEARCH

1Q Fiscal 2014 Conference Call – Prepared Remarks

February 4, 2014

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2014, first quarter financial results 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, Director of Finance and Investor Relations for Arrowhead. Please go ahead Vince.

Vince Anzalone

Thank you, Operator. Good afternoon everyone, and thank you for joining us today to discuss Arrowhead's results for its fiscal 2014 first quarter ended December 31, 2013. With us today from management are President and CEO Dr. Christopher Anzalone, Chief Operating Officer and Head of R&D Dr. Bruce Given, and Chief Financial Officer Ken Myszkowski. Management will provide a brief overview of the quarter and 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 may 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. They represent management's current expectations and are inherently uncertain. Thus, actual results may differ materially. Arrowhead 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 quarterly reports on Form 10-Q for additional matters to be considered in this regard.

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.

During our last conference call in December we discussed key 2013 milestones and how they de-risked our programs and helped shape our current value proposition. That was less than 7 weeks ago, so today we will be more forward looking and focus on our plans for this calendar year. 2013 was indeed a great year for us, but we see even more opportunities for value creation in 2014. We are entering a period of growth marked not only by progress of ARC-520, our candidate against chronic hepatitis B infection, but also by expansion of our clinical pipeline. This is the natural progression for Arrowhead as a maturing

company and sets forth a fundamentally different risk profile than we had just a year ago.

Lets start with capital. Today, we filed a shelf registration statement for up to $200 million dollars in Arrowhead equity securities. Our existing shelf expires this year, and we felt it prudent financial management to maintain an effective shelf registration statement. It also reflects our confidence in ARC-520 and the broader DPC platform. We have a tremendous amount of value yet to unlock, and we believe the best way to maximize that value is to drive clinical development ourselves and not be dependent on early partners. For instance, we are prepared to push ARC-520 all the way through registration. So does that mean that we are raising $200 million dollars right now? No. We have a very strong balance sheet that gives us a runway into 2016 and enables us to fully fund ARC-520 through Phase 2b while pushing 2 additional candidates through clinical proof-of-concept. The new shelf gives us added flexibility to further strengthen our balance sheet at some point in the future and continue independent development beyond that.

The ARC-520 clinical program has provided us increased confidence to drive development internally. In October, we completed planned enrollment in a phase 1 study of ARC-520 in 36 healthy volunteers. The study indicated that ARC-520 was generally safe and well-tolerated at all six dose levels studied. All subjects received their full, assigned dose and there were no discontinuations for adverse events or otherwise. No serious or severe adverse events were reported and laboratory results have not indicated any end-organ toxicity in any subject. We later presented un-blinded Phase 1 data at the HepDART 2013 conference demonstrating that adverse event frequency and severity were the same between placebo and ARC-520.

In November, we applied for regulatory approval in Hong Kong to conduct a single dose Phase 2a study in patients with chronic HBV. The 2 sites and PIs are well known international KOLs that have conducted many HBV trials. Ethics committees from both sites have approved our protocol and all necessary preparations are complete. We are waiting for final approval from the Hong Kong Department of Health to begin the study. Communications have been positive and we believe that we will receive approval and begin treating patients this quarter.

We are currently planning two dose groups of 8 patients each, and we expect it to enroll quickly. We believe both cohorts will be at effective dose levels, and our primary endpoints are safety and tolerability as well as depth and duration of s-antigen knock down. We believe the dosing portion will be complete in the second quarter and we will follow patients until s-antigen levels return to baseline. While we cannot predict how long the duration of effect will be, we believe that top-line data should be available sometime during the summer. Our plan is to provide these data via press release and then present a full dataset at a scientific meeting.

We have a high degree of confidence that the Phase 2a will be successful. The Phase 1 suggested that we have a safe and well-tolerated drug at all doses studied. We saw no dose limiting toxicities, so we do not expect any safety concerns in the 2a. We have generated a substantial amount of data in multiple animal models indicating highly potent knock down. For instance, we published data in the journal Molecular Therapy describing ARC-520 administration in rodent HBV models that led to 3-4 logs, or greater than 99.9%, knockdown of HBV gene products. We have generated data in non-human primates using other siRNA sequences demonstrating similar results. Most recently, we reported deep and durable knockdown in a chimpanzee with chronic HBV. Should the 2a work out as we hope, it will represent a great leap forward in HBV research and potential

treatment. We believe it will be the first time anyone will have demonstrated consistent s-antigen reduction, which is thought to be a critical step in reaching a functional cure.

Based on the Phase 2a data, we plan to move into a multi-dose Phase 2b in the second half of this year. Preparations for this much longer study are underway and they include completion of our second external GMP manufacturing run, chronic GLP toxicology studies in multiple species, site and investigator recruitment, protocol development, and applications for regulatory approval. The Phase 2b will be multi-national and will likely include sites in the U.S., Europe, and Asia. We will be designing the trial to provide a read out on ARC-520's ability to achieve functional cures, among other outcome measures.

As you can see, 2014 is an important year for ARC-520 and we expect substantial value to be created around this program. But 2014 is also about leveraging ARC-520 to de-risk the DPC delivery platform and broadening out our pipeline. So think of ARC-520 as a candidate that drives value directly ***and*** as a proxy for other liver-based candidates. Our friends at Alnylam did the same thing with their TTR program over the past year and a half. We have demonstrated that the DPCs are safe and well-tolerated in humans, and we hope that by the summer we will have demonstrated that they are capable of inducing efficient, deep, and durable gene product knock down in humans. This type of clinical validation will enable shareholders and potential shareholders to ascribe value to new candidates relatively early in development. RNAi is a reliable mechanism and many accept the idea that if you can get a potent siRNA sequence to the right tissue type and the right intracellular space, then you can reasonably expect target gene product knockdown. Once we show that DPCs can do this safely and efficiently in humans, we will have a machine capable of pushing new candidates into the clinic

quickly. These candidates may have a higher probability of success and lower risk profile relative to early clinical candidates using other therapeutic modalities. This is the point when our upside potential expands substantially and we begin to maximize the value we may extract from our broad platforms.

Toward those ends, we expect to have an analyst and investor day at the end of the second quarter to discuss pipeline capabilities and the next candidate. The target and disease area for this next candidate have not been disclosed publicly, but we have said that it is an orphan liver indication. Our current plan is to hold an analyst and investor day to announce the candidate, provide information about the disease area, present preclinical data, and give more guidance about timing for the clinical program. This should be very similar to the event we held last year on ARC-520, and we hope to include key opinion leaders in the disease area. We expect to file an IND for the new candidate in the fourth quarter of this year.

Underneath ARC-520 and the next candidate, we have programs against other targets. We also have large efforts to develop DPC formulations for sub cutaneous administration as well as programs in extra hepatic delivery. We believe these are significant mid- and long-term value drivers for us and we hope to provide additional information around them later in 2014.

With that update, I would now like to turn the call over to our CFO Ken Myszkowski to review our financials for the period. Ken?

Ken Myszkowski

Thank you, Chris, and good afternoon everyone.

As we reported today, our net loss attributable to Arrowhead for the three months ended December 31, 2013 was $10.6 million, or $0.28 per share based on 37.7 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of $4.6 million, or $0.33 per share based on 14.1 million weighted average shares outstanding, for the three months ended December 31, 2012.

Total operating expenses for the three months ended December 31, 2013 were $7.1 million, compared to 5 million for the three months ended December 31, 2012. Research and development related expenses were $4.5 million and $1.7 million for general and administrative expenses. The increase in operating expenses compared to the year ago period, are due to higher drug manufacturing costs, related to ARC-520 in preparation for phase 2 clinical trials, higher clinical trial expense related to the Phase 1 clinical trial for ARC-520, and higher compensation expense, primarily due to increased headcount, as compared to the prior year.

Net cash used in operating activities for the first three months of fiscal 2014 were $7 million, compared with $3.8 million in the prior year period. The change in cash used in operating activities is consistent with the change in operating expenses.

Turning to our balance sheet, our cash balance was $59.7 million at December 31, 2013. Including investments in fixed income securities, our cash and investments balance was $85.5 million at December 31, 2013, compared to $29.8 million at September 30, 2013. The increase reflects the $60 million offering closed in October 2013. Additionally, the Company received cash inflow of $2.8 million from the exercise of warrants and stock options.

Our common shares outstanding at December 31, 2013, were 39 million up 6.5 million from 32.5 million at September 30, 2013. Also, at December 31, 2013, there were 51,291 shares of preferred stock outstanding. These preferred shares are convertible into 10.7 million shares of common stock. Common shares outstanding including the conversion of our preferred shares would be 49.7 million.

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

Chris Anzalone

Thanks Ken. As I mentioned, 2013 was a big year for us but we believe that 2014 offers even more opportunities for real and durable value creation. They include the following:

- This quarter we expect to begin a Phase 2a study of ARC-520 in patients with chronic HBV in Hong Kong.
- Next quarter we expect to complete dosing in the Phase 2a.
- At the end of the second quarter, we expect to have an analyst and investor day to disclose our next candidate. We will discuss the disease, target, preclinical data, and clinical plan.
- In the third quarter we expect to release top line results from the Phase 2a.
- In the fourth quarter, we expect to begin a multi-dose Phase 2b study for ARC-520.
- Also in the fourth quarter, we expect to file an IND for our next candidate.

ARC-520 remains our top priority and primary value driver, but as it moves through the Phase 2a it will also serve as a powerful proof-of-concept for our

broader platforms. I believe that this will represent an important inflection point as shareholder value may then be built simultaneously through the success of ARC-520 as a candidate and via new candidates that enter the clinic relatively de-risked. This becomes a story of leverage and speed. We have developed a machine capable of pushing new candidates into the clinic rapidly that are all built on a validated delivery system. We have all the tools we need to build substantial shareholder value and create new therapies that could positively impact patients worldwide.

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

Operator

Operator opens the call to questions …