ARROWHEAD RESEARCH

2Q Fiscal 2013 CONFERENCE CALL - PREPARED REMARKS

May 9, 2013

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2013, second 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. (Operator instructions) 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 2013 second quarter ended March 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 the call up 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.

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. Thus, actual results may differ materially. Arrowhead undertakes no duty to update any of the forward-looking statements discussed on 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. As you know, our mission is to create powerful new therapies that address real health problems. Raw innovation and execution toward commercial development are the necessary engines driving us toward this goal, and we made substantial progress on both over the past quarter. Of course these engines require capital to run, and we recently announced a $36 million financing from a syndicate of high quality healthcare investors. This gives us a cash runway into 2015, and now we are properly funded to execute on our strategy and accelerate the development of our RNAi-based treatments. We believe our team and technological platform are second to none, and we now have the capital to properly fuel that team and technology.

Let's take a closer look at the financing and what we believe it means. This is the first time in the company's history that Arrowhead has been able to attract this amount of capital and this high quality of fundamental investors. RA Capital led the round with a syndicate that included Special Situations Fund, Camber Capital, Sabby Capital, Aquilo Capital, Sphera Global Healthcare Fund, and investor Jim Mellon. These funds conducted extensive due diligence over several months, and while bringing them in was not an ultimate goal in and of itself, this financing is a strong indication that some very sophisticated investors have confidence in our technology, plan, and ability to create value. The structure of the financing is also important because it is shareholder-friendly. The offering was priced at-the-market and included no warrant coverage. This is a welcome step forward and we believe it is unique for a company our size. This financing is a turning point for what it represents and for what it enables us to achieve.

We now have the resources to reach several important near-term milestones and critical data points. These include:

1. Filing with Australian authorities this quarter to launch a Phase 1 study in healthy volunteers with our hepatitis B candidate, ARC-520;
2. Data from the ARC-520 phase 1, establishing a safety profile for the candidate and representing the first human data with the DPC delivery platform;
3. Regulatory filing in Hong Kong in calendar Q4 2013 for a single dose Phase 2a with ARC-520 in chronic HBV patients;
4. Data from the phase 2a study;
5. Completion of long term GLP toxicology studies and initiation of a multi-dose phase 2b study of ARC-520, planned for the second half of 2014; and

6. Completion of preclinical work to designate at least one new RNAi clinical candidate.

In addition to these discrete goals, throughout 2013 and 2014 we plan to release updates on our pipeline and underlying technologies through publications, scientific meetings, investment conferences, webinars, and analyst/investor events. We also anticipate data being available from the ongoing phase 1 study of Adipotide, our anti-obesity candidate, being conducted at MD Anderson Cancer Center. However, since we do not fund or control that study, I have not included it in this list.

These are important near-term value drivers because they serve to de-risk the ARC-520 program specifically, and the DPC delivery system more broadly. The ARC-520 clinical program, which we will discuss in more detail later in the call, is designed to provide us with a relatively early efficacy read-out. Over the next 12-18 months, we expect to have a good idea about whether the candidate will have a similar safety and efficacy profile in humans as we have seen in animal models. If these results reflect the results in two established rodent models and the HBV-infected chimpanzee, we expect ARC-520's commercial risk profile, and therefore value, to change dramatically. Clinical proof of concept could also de-risk the broader DPC platform, and drive value into the base technology and follow-on candidates.

I would like to move on to a review of the steps we've taken and the progress we've made in recent months. As we discussed on our last conference call, we view 2013 as a demonstration year. We spent 2012 building out the assets and capabilities we acquired with the Roche transaction, and much of this was not or could not be publicized. However, that work positioned us to generate and actively

publicize the type of clinical and non-clinical data that represent valuable proof-of-concept.

For example, we published a paper in the journal Molecular Therapy detailing the non-clinical development of ARC-520. It included key data demonstrating a new way of treating chronic hepatitis B virus infection by silencing the entire HBV genome. With this approach, we believe achieving a functional cure is possible. The data showed multi-log reductions in hepatitis B viral DNA and proteins, including s-antigen, lasting over 30 days after a single injection. We do not believe any group has ever shown reductions as rapid or as deep as this. The s-antigen reductions were potentially most important. It is widely believed that s-antigen reduction, which no current therapy is capable of consistently inducing, is the necessary step in achieving a functional cure. We believe the s-antigen reductions that ARC-520 induced were substantially more pronounced than anything ever demonstrated to date. More broadly, the data demonstrated that the DPC delivery system is capable of extremely efficient gene silencing that should also be applicable to a variety disease targets.

This work was done in two well-established rodent models that are accepted as appropriate and reasonably predictive. However, we believed that we could further de-risk ARC-520 by demonstrating safety and efficacy in a primate before moving into human studies. Chimpanzees are naturally susceptible to the same hepatitis B virus as humans, and their response to infection is very similar. Chimpanzees were used in the development of HBV vaccines and are thought to be predictive of human safety and efficacy responses. We became aware of 2 chimpanzees with chronic HBV infection that could be studied, and we were provided access to one of them.

In many ways, this animal was a very good model. Of course as a chimpanzee, its physiology is quite similar to that of humans, and it was infected with HBV at a young age. However, there were 2 areas that made it a very challenging model. First, it presented with extremely high viremia and antigenemia. We believe that its viral load and HBV protein levels were likely several orders of magnitude higher than we ever expect to see in most human patients, so the bar for reducing viral and antigen levels was quite high. Second, the animal had a mutant form of HBV that is likely resistant to one of the 2 siRNA sequences in ARC-520. Therefore, we were fighting a very high infection with only half our dose of ARC-520 being effective. We viewed this as a worst-case scenario.

Even so, after a low dose of ARC-520 we demonstrated a level and speed of viral and protein reduction never reported. We identified a well-tolerated dose of ARC-520 leading to a 95% reduction in circulating viral DNA. More importantly, ARC-520 led to approximately 90% reductions in hepatitis e-antigen and s-antigen. These data support previous findings in rodent models and aid in predicting a therapeutic dose range to be evaluated in upcoming clinical trials. We did not see any toxicity signals that we can attribute to the drug, so we believe that ARC-520 will have a comfortable therapeutic index. This study is ongoing and we will release additional data in the future.

Following the release of those initial data, we hosted a well-attended analyst and investor event in New York City, to discuss our hepatitis B program. Panel members for the event included company management and key opinion leaders in HBV: Dr. Robert Gish, noted hepatologist and Arrowhead Clinical Advisory Board Chairman; and Joan Block, Executive Director and Co-founder of the Hepatitis B Foundation. The live event was also webcast and if you missed it an archive is available on the Arrowhead website.

Over the past few months, we expanded our intellectual property protection through two new patent allowances covering the DPC delivery system. The first was for a next generation protease-sensitive masking technology that enables new DPC constructs that can be engineered for long circulation times and improved tissue-targeting characteristics. DPCs with this new masking technology have been shown to be highly potent upon subcutaneous administration and may enable new therapeutic targets including oncology. The second patent allowance, which we announced earlier this week, specifically protects the use of targeted melittin or melittin-like peptides to facilitate delivery of siRNA conjugates to hepatocytes. This new patent protection covers the composition of the DPCs used in ARC-520 and runs until 2031. Needless to say, we have long-lasting IP protection for this candidate.

Lastly, we made progress towards completing the final steps required to file a regulatory submission for first-in-man studies of ARC-520. We completed GMP manufacturing of clinical drug supply for phase 1 and phase 2a, and completed GLP toxicology studies with a final study report expected shortly. We have recruited the clinical site and investigator, and we are now in the final stages of preparing the regulatory filing for submission during this quarter.

With that update, I would now like to turn the call over to our COO and Head of R&D Dr. Bruce Given to review the ARC-520 clinical program in a little more detail. Bruce?

Bruce Given

Thanks Chris, and hello to everyone on the call today.

Before I talk about our clinical trial strategy and timeline, I want to first review why we believe HBV is an attractive commercial opportunity and a disease that is well suited for an RNAi-based intervention. HBV is the world's most common serious liver infection, with an estimated 350 million patients worldwide that are chronically infected. There are thought to be approximately 2 million patients in the U.S., 14 million in Western Europe, over 100 million in the Asia Pacific Region, and another 220+ million throughout the rest of the world. HBV can lead to cirrhosis of the liver and is responsible for 80% of primary liver cancers globally. The annual death toll for HBV is estimated as high as one million. So this is truly a global disease that imposes an enormous health burden, and death toll, on both the developed and developing world.

The goal of ARC-520 is to provide a functional cure – an immune clearant state characterized by hepatitis B s-antigen negative serum with or without sero-conversion. This goal leads to my next point about why we believe RNAi is uniquely suited to address HBV. Current treatment options include interferon, which is difficult to use due to highly disruptive side effects, and nucleotide or nucleoside analogues, referred to collectively as NUCs. The best NUCs are very good at suppressing viremia, the production and release of new viral particles, but are not capable of directly suppressing the production and release of viral proteins including s-antigen and e-antigen. Neither interferon nor NUCs provide meaningful rates of functional cure. Many experts in the field believe that addressing both viremia and antigenemia is required to obtain a functional cure. RNAi in general, and the siRNAs in ARC-520 specifically, act in a fundamentally different way than NUCs. They intervene upstream at the point of DNA transcription and can deeply knockdown all HBV gene products, including proteins and the viral intermediates necessary to produce viral DNA. No other mechanism

and, to our knowledge, no other drug currently used or in development has been able to reliably do this.

Moving on to our clinical program for ARC-520. As Chris mentioned, we are making final preparations for a regulatory submission to begin a phase 1 study. This will be a single ascending-dose study in approximately 42 healthy volunteers in Australia. It is designed to provide a standard assessment of safety and tolerability. We are on schedule to meet our stated goal of a submission this quarter and dosing will begin soon after we receive ethics and regulatory approval. We believe the study will be complete and data available in Q4.

We are planning to follow this with a single dose phase 2a study in chronic HBV patients in Hong Kong. The phase 1 study will establish a safety profile relatively rapidly, and should enable us to begin this phase 2a study in patients at a therapeutic dose level, thereby accelerating the path to meaningful results. Our plan is to apply for ethics and regulatory permission in the fall. In addition to assessing safety and tolerability in patients, we will follow measures of viral load and antigenemia, and also determine depth and duration of effect.

Historically, data from chimpanzees with chronic HBV have been very predictive of dosing and response in humans, so we are eager to see the readout from our phase 2a study.

If successful, we are planning to conduct a multi-dose phase 2b study beginning in the second half of 2014. In order to meet that timeline, we will have to, among other steps, complete a multi-dose GLP toxicology study, which is planned to initiate next quarter.

Following the next twelve months of work we should have a well-rounded data package for ARC-520 that includes extensive non-clinical data, volunteer and patient safety and tolerability, and critically, we expect to have an early readout on the extent and duration of our ability to knock down production of new virus and key viral proteins. We think these goals are aggressive but achievable.

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, Bruce, and good afternoon everyone.

As we reported today, our net loss attributable to Arrowhead for the three months ended March 31, 2013 was $6.8 million, or $0.41 per share based on 16.5 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of $5.3 million, or $0.50 per share based on 10.7 million weighted average shares outstanding, for the three months ended March 31, 2012.

Total operating expenses for the three months ended March 31, 2013 were $5.4 million, compared to 4.9 million for the three months ended March 31, 2012.

Net cash used in operating activities for the first six months of fiscal 2013 were $8.3 million, compared with $6.9 million in the prior year period.

Increased operating expenses and cash used in operating activities reflect pre-clinical requirements and final IND-enabling steps, including GMP manufacturing and GLP toxicology, for our HBV program.

Turning to our balance sheet, our cash position was $3.3 million at March 31, 2013, compared to $3.4 million at September 30, 2012. During the first half of the fiscal year, cash outlays for R&D were $6.1 million, and cash used in G&A were $2.7 million. Cash inflows during the first half of the fiscal year included $7.1 million from the sale of equity securities in public offerings, $400,000 in revenue, and $1.2 million in proceeds related to the sale of our former subsidiary, Unidym.

Our shares outstanding at March 31, 2013, were 17.0 million up 3.4 million from 13.6 million at September 30, 2012. Including the $36 million offering closed last week, our common shares currently outstanding is 31.3 million, and would be 36.7 million shares inclusive of the conversion of the preferred shares issued.

With that brief overview, I will now turn the call over to Chris for concluding remarks.

Chris Anzalone

Thank you Ken.

You've heard me talk about the concept of "multiple shots on goal" many times over the last few years. This approach is designed to open opportunities, mitigate shareholder risk, and search for value. Once that value is identified, it is critical for a company to be nimble and move quickly to fully support it. ARC-520 and the flexible platform that enables it together represent that value and our company is fully aligned behind them. This is where we are today; this is when our work

acquiring technologies and building capabilities begins to pay off; and this is why it is a very exciting time at Arrowhead.

We believe that ARC-520 is a well thought out candidate from a market and technological standpoint. Approximately 1 out of every 20 people on the planet is infected with HBV and there is no cure. Our approach is unique in its focus on silencing the entire HBV genome, and it is the only therapy in development we are aware of that could potentially lead to a functional cure. As they say in billiards, there is still a lot of green between the ball and the pocket, but we have first mover advantage.

In addition, we have substantially de-risked the program. We have a good understanding of the safety profile after many studies in non-human primates and rodents. The final report from the GLP toxicology studies will be available shortly and we have not seen any surprises to date. We have a good understanding of potential efficacy after studies in 2 rodent models and most recently a chimpanzee with chronic HBV infection. Finally, our timing is attractive as we will file this quarter to begin human studies.

For all its promise, ARC-520 is not our only value driver. The underlying DPC siRNA delivery system is extremely versatile and as ARC-520 progresses through the clinic we believe it will drive value in the platform that enables it. We have a team of dedicated scientists that strive everyday to make the DPCs even safer and more potent, including recent advances with constructs capable of sub-cutaneous administration and tumor targeting. And now, after our recent financing from a new long-term supportive base of institutional investors, we have the capital needed to accelerate our development programs.

We are committed to raising the profile of the company and firmly believe we are well on our way to becoming a leading developer of RNAi therapeutics. As I mentioned, we anticipate several important milestone events over the next twelve months that we believe will further that goal.

Thank you for your interest in Arrowhead Research and I would now like to open the call to your questions. Operator?

Operator

Operator opens the call to questions …