ARROWHEAD RESEARCH

3Q Fiscal 2013 CONFERENCE CALL - PREPARED REMARKS

August 7, 2013

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2013, third 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 2013 third quarter ended June 30, 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. 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.

We have many new shareholders, so I would like to take a moment and review key events that shaped our capabilities, business model, and priorities. In October of 2011 we acquired Roche's RNAi therapeutics business, which included the Dynamic Polyconjugate, or DPC, delivery system that we use in our hepatitis B drug candidate, ARC-520. As many of you know, Roche invested over half a billion dollars to create this unit. They built it in a manner that only a large pharmaceutical company is capable of: they invested a very large amount of capital and systematically acquired technologies, licensed expansive IP, attracted leading scientists, developed new technologies internally, and built state-of-the-art

facilities. At a time when the markets were questioning whether RNAi could become a viable therapeutic modality, we saw great promise in the technology broadly and the quality of what Roche built specifically. The acquisition provided us with three primary silos of value:

(1) Broad freedom to operate within three siRNA formats: these are canonical, meroduplex, and dicer substrate siRNA structures;

(2) What we believe to be best-in-class small RNA delivery system, the targetable DPC platform; and

(3) A state-of-the-art R&D facility in Madison, Wisconsin and a large team of scientists who are experienced in RNAi and siRNA delivery.

This was and is a powerful combination indeed. It provided us with the tools we needed to build an independent and broad RNAi company. We believe we are the only company with access to all three siRNA structures and this enables us to optimize the RNAi trigger on a target-by-target basis. Our DPC delivery system enables us to deliver siRNA efficiently to hepatocytes and non-hepatic tissues in a highly specific manner. Our R&D team and facility enable rapid innovation and drive to the clinic, as evidenced by ARC-520. As we look at the RNAi space, we do not see any company with as powerful and complete a combination of assets and capabilities as ours.

We have made great strides since the acquisition. We brought ARC-520 into the clinic and made important advances in the DPC delivery technology. This includes new generations of DPCs capable of inducing deep and durable gene knockdown with various constructs designed for both IV and subcutaneous administration. A key to DPC's potency, and one of its differentiating qualities, is a polymer backbone designed to induce efficient endosomal escape. This allows more of the siRNA to get into the cytosol where it can engage the cell's RNAi machinery. We

have also taken advantage of the fact that DPCs are targetable and made substantial progress toward extra-hepatic delivery.

Delivering outside the liver is important for maximizing the value of DPCs and to continue to differentiate Arrowhead from other RNAi players. Toward those ends, we have active programs to identify and evaluate targeting ligands that may be used with DPCs. As part of this initiative, in April 2012 we acquired Alvos Therapeutics, a privately held company that licensed a large platform of proprietary human-derived homing peptides from MD Anderson Cancer Center. This library contains peptides discovered through screening in human patients, and we were interested in determining whether we could use them to target DPCs.

As our DPC platform continued to develop and we pushed ARC-520 toward the clinic, it became clear that these would be our primary near- and long-term value drivers. As such, we have lined up our resources behind them and our focus is entirely on pushing ARC-520 through the clinic and developing additional DPC-enabled RNAi therapeutics. It has been said that for a biotech company to succeed, it needs to be number 1 or 2 in the world in something. We believe that DPCs are the most efficient and flexible siRNA delivery system in the industry, and clinical data from ARC-520 will make the platform tangible. Of course it is still early, but we believe that ARC-520 has the potential to represent a paradigm shift in the treatment of Hepatitis B.

We look at our priorities from the perspective of ARC-520 outward. In other words, think of a set of concentric circles, with our near-term opportunities represented by the inner circles and longer-term opportunities represented by outer circles. ARC-520 is the bullseye and our top priority is to ensure that it moves forward and is properly resourced. Just outside that are IV administered liver-

targeted therapeutics. These could use the same DPC formulation as ARC-520 and would be straightforward to develop and substantially de-risked, particularly once we have human safety data later this year from ARC-520. The next circle is subcutaneous administered liver-targeted therapeutics. We presented data from this program last fall and continue to make good progress. The outer circle is extra-hepatic targets, including oncology. We are comfortable that all of these areas are now properly resourced and hope to provide guidance on timing later this year.

Sharpening our focus on DPC-enabled RNAi therapeutics also means making difficult decisions about closing some programs and deemphasizing others. We have taken multiple disciplined steps to ensure that our capital is allocated to maximize shareholder value. These include the following:

First, we are not pursuing internal development of peptide-drug conjugates with the Alvos targeting library. As you may recall, these peptides could potentially be used to target traditional drugs in addition to DPCs, but our only interest for internal development revolves around possible DPC targeting to extra-hepatic tissues. We still see potential value in other drug conjugates, but any development and commercialization outside RNAi will be via partnerships such as the one we have with Shire.

Second, we have decided not to pursue additional candidates based on the RONDEL delivery platform. In our 10Q today, we disclosed that IP and licenses related to the cyclodextrin-based polymer system have been returned to CalTech, the licensor. Our data indicate that DPC delivery is a far superior solution so we can no longer justify the expense of further development and maintaining the RONDEL patent portfolio. For this reason, we have also decided not to advance

the CALAA-01 candidate into a Phase 2. Arrowhead still maintains a financial interest in the Cyclosert delivery platform, the CRLX101 drug candidate, and follow-on candidates through our out-license agreement with Cerulean Pharma.

Third, while the Adipotide preclinical data in rodents and non-human primates across multiple laboratories and published in high quality peer-reviewed journals were compelling; the program is now outside Arrowhead's core focus. As you may recall, MD Anderson is conducting and fully funding the Phase 1 trial, which began last year. Arrowhead will continue to monitor progress of the trial, but we are not currently expending any internal resources to the program. Patients continue to be recruited and treated, and as results come in, we can assess whether internal development resources are warranted or it may be an attractive licensing candidate.

I would now like to turn the call over to our COO and Head of R&D, Dr. Bruce Given, to discuss the ARC-520 clinical program. Bruce?

Bruce Given

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

As you know, ARC-520 is our clinical candidate against chronic hepatitis B infection. HBV is the world's most common serious liver infection, with an estimated 350 million patients worldwide that are chronically infected. The goal of ARC-520 is to provide a functional cure, which is an immune clearant state characterized by hepatitis B s-antigen negative serum with or without sero-conversion. In March, we hosted an event to discuss the hepatitis B treatment landscape and our ARC-520 program in more detail. An archive of the webcast is

available on the Arrowhead website and I would recommend viewing it if you want more information about HBV.

We recently initiated a Phase 1 study of ARC-520 following successful completion of the Clinical Trial Notification regulatory process in Australia. The objectives of the study are to characterize the safety profile of ARC-520, determine the maximum tolerated dose, and evaluate pharmacokinetics. It is a single-center, randomized, double-blind, placebo-controlled, single dose-escalation, first-in-human study of ARC-520 administered intravenously to healthy adult volunteers and is being conducted in Melbourne, Australia. Each dose cohort includes 6 subjects randomized at ratio of 1 to 2 to receive a single intravenous injection of either placebo or ARC-520, respectively. We have completed dosing of 8 volunteers and expect 4 additional subjects to be treated today. Therefore, by the end of today, we expect to have completed the first 2 of 6 cohorts. The trial is moving quickly, and we are on schedule to meet our stated goal of completing the trial in the fourth quarter.

Following completion of the Phase 1, we plan to apply for ethics and regulatory permission to initiate a Phase 2a study in chronic HBV patients in Hong Kong. Many experts in the field believe that addressing circulating s-antigen is required to obtain a functional cure of chronic HBV. So, we have been working on finalizing the design for this trial that will provide a readout of ARC-520's ability to reduce the production s-antigen. We will provide more guidance on trial design in the future, but our current thinking is that it will include patients that are currently being treated with a nucleotide or nucleoside analogue. We believe a patient population with adequately controlled viral load but uncontrolled antigenemia may provide us with a clear signal of ARC-520's activity. We will follow not only the depth of antigen reduction but also duration of effect. The final

trial design is being developed with guidance from our clinical advisory board, chaired by Dr. Robert Gish and including Drs. Stephen Locarnini, C.L. Lai, and Johnson Lau.

Following the Phase 2a, we are planning to conduct a multi-dose Phase 2b study. This will be a multi-national study including a planned US IND filing. We are currently working on scale-up of manufacturing to produce the clinical drug supply and we are initiating a nine-month multi-dose GLP toxicology study to support our goal of initiating the Phase 2b in the second half of 2014.

Moving beyond ARC-520, as Chris mentioned previously we are actively working on designating our next clinical candidate that will use DPC delivery. We have consulted with our expert advisors on targets of interest and will convene a meeting with the full board next week. We will provide more guidance on what the targets are as we move forward and intend to have at least one candidate nominated next year. One of the attractive features of RNAi and of DPCs specifically is once we have established the safety profile in man for the delivery system, additional targets and candidates can be studied rapidly. We believe the Phase 1 trial of ARC-520 will provide us with the data we need to accelerate the development of our pipeline and bring additional candidates into the clinic.

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 June 30, 2013 was $6.1 million, or $0.23 per share based on 26.1 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of $8.0 million, or $0.71 per share based on 11.2 million weighted average shares outstanding, for the three months ended June 30, 2012.

Total operating expenses for the three months ended June 30, 2013 were $6.4 million, compared to 6.9 million for the three months ended June 30, 2012.

Net cash used in operating activities for the first nine months of fiscal 2013 were $13.6 million, compared with $10.8 million in the prior year period.

The increase in cash used in operating activities reflects final pre-clinical requirements, including GMP manufacturing and GLP toxicology, to enable our HBV candidate, ARC-520, to enter clinical trials.

Turning to our balance sheet, our cash and short-term investments were $33.1 million at June 30, 2013, compared to $3.4 million at September 30, 2012. The increase in our cash balance reflects the $36 million offering closed in May 2013. During the first nine months of the fiscal year, cash outlays for R&D were $10.1 million, and cash used in G&A were $4.4 million. Cash inflows during the first nine months of the fiscal year included $42.5 million from the sale of equity securities, $500,000 in revenue, and $1.2 million in proceeds related to the sale of our former subsidiary, Unidym.

Our shares outstanding at June 30, 2013, were 31.3 million up 17.7 million from 13.6 million at September 30, 2012. Common shares outstanding including the conversion of our preferred shares would be 36.7 million.

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

Chris Anzalone

Thanks Ken. We have made substantial progress in recent months. As I mentioned, we have streamlined our business behind the DPC platform and our first candidate built on it, ARC-520. A number of accomplishments speak to that progress and serve to fuel future developments, including the following:

1. We strengthened our balance sheet with a $36 million financing from a syndicate of high-quality biotech investors. This provided sufficient capital to fund development into 2015;
2. We presented DPC and ARC-520 data at several scientific and investor conferences;
3. We completed the Clinical Trial Notification regulatory process in Australia for ARC-520;
4. We initiated a Phase 1 trial of ARC-520 and by the end of today we will have completed dosing of the first 2 of 6 planned cohorts; and
5. We assembled and consulted with an advisory board on additional liver targets.

What does this mean for the future? We have several important near-term milestones.

We anticipate data from the ARC-520 phase 1 in Q4, establishing a safety profile for the candidate and representing the first human data with the DPC delivery

platform. This is an important de-risking event for ARC-520 and also represents a broader de-risking of the entire DPC platform.

We will present a full dataset from our chimpanzee study at the 64th annual meeting of the American Association for the Study of Liver Diseases in November. Dr. Robert Lanford, who conducted the study at the Texas Biomedical Research Institute, will present the data.

We are on schedule for regulatory filing in Hong Kong in Q4 for a single dose Phase 2a with ARC-520 in chronic HBV patients. This will be a dose escalation study and we believe that we may start at an effective dose level and, therefore, begin to generate meaningful data immediately. Our goal is to complete the phase 2a study in the first half of 2014.

As Bruce mentioned, completion of long term GLP toxicology studies and initiation of a multi-dose phase 2b study of ARC-520 are planned for the second half of 2014. And lastly, we intend to complete preclinical work to designate at least one new RNAi clinical candidate in 2014.

I spoke in the past about 2012 being a building year, when we focused inward on the assets acquired from Roche, and 2013 being a demonstration year where we show evidence of the great potential of our RNAi platform and pipeline. I believe the past several months have borne that out and the following key points describe where we are today as a company:

We have a world-class team that has demonstrated its ability to rapidly innovate and meet aggressive development timelines. Our set of proprietary technologies enables us to address a wide variety of indications in a uniquely powerful way and

we have published data demonstrating a level of gene knockdown not seen before. We have a first candidate in ARC-520 that addresses a disease that infects approximately 1 in 20 people on the planet and has no cure. The preclinical data with this candidate in multiple animal models, including a chimpanzee, has surpassed anything we are aware of and has fundamentally de-risked the first candidate and underlying platform. We have a market that is now accepting of RNAi as a therapeutic modality where it once doubted its viability. And lastly, we have sufficient resources to enable us to push our platform and candidates forward quickly.

We are now focused on accelerating this demonstration phase and pushing it into clinical demonstration. We expect to have regular clinical readouts over the next several quarters designed to further de-risk our programs and demonstrate substantial shareholder value. We firmly believe that we are becoming a recognized leader in RNAi therapeutics and thank all of our shareholders for supporting Arrowhead Research.

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

Operator

Operator opens the call to questions …