ARROWHEAD RESEARCH

4Q Fiscal 2012 CONFERENCE CALL - PREPARED REMARKS

December 20, 2012

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2012 fourth quarter and year-end 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 2012 fourth quarter and year ended September 30, 2012. 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. Without limiting the generality of the foregoing, words such as may, will, expect, believe, anticipate, intend, could, estimate, or continue, or the negative or other variations thereof, or comparable terminology, are intended to identify forward-looking statements. In addition, any statements that refer to projections of Arrowhead's future financial performance, trends in its businesses, or other characterizations of future events or circumstances are forward-looking statements. Forward-looking statements represent management's current expectations and are inherently uncertain.

You should also 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 on our call today. Let me start by discussing the recent announcement that we signed a Collaboration and License Agreement with Shire to use our Homing Peptide platform. This is the second targeting agreement we have made with

pharmaceutical companies, the first being with Merck in August, since we acquired the platform in April of this year. The agreement with Shire includes research funding for Arrowhead to screen our library of human-derived Homing Peptides and identify targeting agents that can preferentially deliver a therapeutic payload to a specific undisclosed tissue type. Shire will then be responsible for clinical development and commercialization of a peptide-drug conjugate or PDC using our targeting peptide and one of their therapeutic agents. We are eligible to receive up to $32.8 million in milestone payments, plus additional milestones if Shire seeks regulatory approval for a second indication. We will also receive royalties on the sale of such products.

We are encouraged by the interest from companies of this caliber and see this as an important value driver in four primary areas.

First, it provides validation for the technology. Working with Merck and Shire in two very different fields so soon after we acquired the platform speaks to the breadth of the technology and suggests that established pharmaceutical companies see value in the platform.

Second, there is direct economic value in the partnership because Shire will provide research funding, milestone payments, and royalties as the program moves forward.

Third, Shire funding will help to pay for development of targeting that we may use outside the partnership. This is truly leveraging a specific collaboration to build value for the entire platform.

And, fourth, this deal demonstrates an evolution of partnership structure and economics. Our first collaboration with Merck left negotiation of further development and commercialization economics until after evaluations are complete. The Shire partnership contains more concrete development plans and economics, and we believe it is a positive step toward larger deals.

Moving on, we are making progress with our product pipeline. This currently includes an oncology RNAi therapeutic, CALAA-01, an anti-obesity PDC, Adipotide, and our newest drug candidate ARC-520 for the treatment of chronic hepatitis B virus (or HBV) infection. During the fiscal fourth quarter of 2012 and since our last conference call, we have seen a number of accomplishments, including:

1. As mentioned, we executed a Collaboration and License Agreement with Shire for peptide-targeted therapeutics
2. Entered into an Evaluation Agreement with Merck for a monoclonal antibody candidate;
3. Began dosing patients with anti-obesity treatment Adipotide® in a phase 1 clinical trial;
4. Completed patient dosing in a phase 1b trial of CALAA-01;
5. Enrollment of a randomized phase 2 clinical study of partnered candidate CRLX101 (formerly IT-101) in non-small cell lung cancer was completed by licensee Cerulean Pharma, with overall survival data expected in early 2013;
6. Received additional patents on the DPC siRNA delivery system;
7. Presented data on new DPC subcutaneous formulation showing 99% target gene knockdown in non-human primates without observed toxicity;
8. Published studies in the journal Nucleic Acid Therapeutics demonstrating high levels of knockdown in non-human primates using cholesterol-conjugated siRNA, DPCs, and a novel co-injection strategy;
9. Established a Clinical Advisory Board for hepatitis B candidate ARC-520 and named prominent hepatologist Dr. Robert G. Gish Chairman;

10. Submitted a pre-IND data package and completed a pre-IND meeting with the FDA on ARC-520;
11. Advanced ARC-520 into final IND-enabling steps, with IND filing expected in Q2 2013;
12. Strengthened the balance sheet through equity financings with gross proceeds of $10.5 million.

I'd like to highlight a few items in more detail. With the Shire deal fresh in our minds, this is good time to provide some guidance on our strategy for the Homing Peptide platform and how we plan to utilize it. Our acquisition of Alvos Therapeutics in April was in large part intended to drive value for our targeted RNAi therapeutics programs. Acquisition of the Roche RNAi business gave us extremely broad and powerful capabilities in targeted RNAi therapeutics. We believed that our capabilities could expand exponentially if we had access to a variety of targeting ligands capable of guiding RNAi therapeutics to multiple tissue types in a highly specific manner. For this primary reason, we acquired the Homing Peptide library, which contains over 42,000 targeting sequences and has the potential to allow preferential delivery to more than 30 tissue types. The combinatorial derivations of the huge number of potential gene targets and this variety of homing peptides is staggering. It provides us with a virtually limitless set of potential therapeutics. Of course we could never create even a fraction of these potential drugs; but it gives us options, which is critical in the difficult world of drug development. It also drives value creation by enabling partners to build therapeutics that we do not intend to develop internally.

Almost as a byproduct of developing targeted RNAi therapeutics with the homing peptide library, we are developing the ability to attach more traditional drugs

directly to these peptides and enable peptide-drug conjugates. This is strategically well-timed in light of the recent success of antibody-drug conjugates.

During the last two quarters, our internal work with this platform has followed these ideas. We continue to explore the use of various targeting peptides for RNAi therapeutics and small molecule drugs.

We will now move on to a discussion of ARC-520, our candidate for the treatment of chronic Hepatitis B infection. It is our first RNAi therapeutic utilizing the DPC delivery system. This program was initiated by Roche and was at an early stage of development at the time of our acquisition. I am extremely pleased with the speed of development over the past twelve months and we are on track to file an IND in the second calendar quarter of 2013. This has served as a compelling proof of concept for the DPC platform, it is an example of how quickly our team in Madison is able to innovate and drive to the clinic, and it is an exciting candidate addressing a large underserved world market.

We released a white paper, which is available on our website, about the HBV landscape and an introduction to our HBV development programs, but we have not discussed many details about ARC-520 to this point. Data from several of the supporting studies are the subject of scientific manuscripts that have been submitted for publication in peer-reviewed journals. We see this candidate as a potentially significant value driver for Arrowhead with several short and medium-term milestones that may serve as catalysts. Although we will not discuss in detail the data that are pending publication, we would like to talk more about the ARC-520 candidate and the rationale for using the RNAi mechanism to target HBV.

According to the World Health Organization, 360 million people globally are chronically infected with HBV, of which between 500,000 and one million people die each year as a result of hepatocellular carcinoma (or HCC), cirrhosis of the liver or liver failure caused by HBV. Progress on effective treatments has lagged behind those for hepatitis C in part because HBV biology is complex and not entirely understood.

Infected hepatocytes release new copies of the virus as well as viral proteins. It is thought that viral proteins can immunosuppress infected individuals and keep them from clearing the virus. Therefore, it is theorized that knocking out the release of viral proteins could enable the patient's immune system to come back up and clear the infection. Unfortunately there is not currently a reliable way to do that. The current standard of care for treatment of chronic HBV is a daily oral dose of nucleotide and nucleoside analogs (or NUCs) or a regimen of interferon injections 2 to 7 times weekly for approximately one year. NUCs are generally well tolerated, and they are effective at blocking the release of new virions, so infected patients are no longer contagious. However, they do virtually nothing against viral proteins so patients are generally on the drug for life and still at risk for HCC, cirrhosis, and other conditions associated with chronic HBV infection. Interferon therapeutics can result in a functional cure in around 10% of patients, but are often associated with significant side effects, including severe flu-like symptoms, marrow suppression, and autoimmune disorders.

This is the opportunity we step into: a huge number of potential patients and sub-optimal treatment options. We believe that our novel therapeutic approach has the potential to effectively treat or provide a functional cure for chronic HBV infection with less side effects than current treatments. Lets talk about why we believe this.

The cellular pathways leading to the export of new virions and viral proteins both include transcription of viral mRNA. Therefore, if a therapy can turn off the entire HBV genome by knocking out all viral mRNAs, it could silence the export of new virions and viral proteins in a single step. If this is done, a patient could become less contagious by decreasing viral load and potentially experience a functional cure if viral proteins are sufficiently reduced. RNAi as a mechanism is uniquely capable of such an action, so what is required is the right siRNA sequence or multiple sequences and a delivery system that will effectively and safely target hepatocytes.

Our scientists screened over 100 siRNA sequences that could in theory silence the entire HBV genome. 2 of those sequences appeared to be most effective and together corresponded to over 99% of known HBV genomes stored in GenBank. ARC-520 includes both sequences and, importantly, leads to broad mRNA suppression, including those of s-antigen, viral polymerase, the core protein that forms the capsid, and the e-antigen. These are therapeutically significant and, to our knowledge, no other potential therapy has this type of coverage.

Nonclinical efficacy data in multiple murine HBV models demonstrated that ARC-520 is capable of multi-log knock down of s-antigen, multi-log knock down of HBV DNA , and e-antigen to the limit of detection after a single injection. Pharmacologic effects persist for approximately 1 month after a single dose. It is widely believed that a similar persistent decrease in these parameters in patients could result in a beneficial outcome and the possibility of functional cure. If these data repeat in humans we believe that we could capture substantial market share. Also importantly, we believe we could significantly expand the treated HBV market. The majority of chronic HBV carriers are currently treated with “wait and see” strategies rather than with drugs. We believe that if a therapeutic existed with

reliable rates of functional cure and a tolerable safety profile, a substantially larger percentage of infected individuals would become patients. This could be a very large number with a worldwide population of infected individuals greater than 360 million.

We submitted our Pre-IND data package and subsequently had a productive meeting with the FDA regarding our development plan. We are initiating the GLP toxicology studies required to progress to human studies. We intend to provide additional guidance on the expected development timelines and our clinical trial strategy as we approach our filing in the second quarter of 2013.

Of course key to our rapid progress with ARC-520 has been the efficiency and tolerability of the DPC delivery system. It is modular so we have many ongoing programs to expand on the library of components and proprietary chemistries in order to broaden its use. Notably, a new DPC construct was developed for subcutaneous injection that produces high-level gene knockdown with a low required dose and no observed toxicity. Studies have demonstrated knockdown of 99% in monkeys after a single injection of 1 mg/kg, >90% at 0.5 mg/kg, and 80% in mice at 0.05 mg/kg. The duration of effect has been observed at more than 7 weeks after a single injection. There were no changes in clinical chemistry markers and no changes in hematology. Subcutaneous dosing is perceived as more desirable than i.v. dosing for many indications, so this step forward has expanded the number of potential markets we may address.

Staying with our RNAi programs, we completed the Phase 1b for CALAA-01 in the last fiscal quarter. This is an RNAi candidate developed to treat solid tumors. We were pleased that it met its primary endpoints in determining a maximum tolerated dosing schedule and of course we were also pleased that it was the first

RNAi therapeutic to demonstrate protein and mRNA knockdown in humans and the first to demonstrate RNAi in humans outside the liver after systemic delivery. Over the past quarter and beyond, we have been studying its efficiency and dynamics against the DPCs. As we better understand its advantages and disadvantages compared to the various DPC constructs, we will be able to make a more informed decision on how to move forward with CALAA-01 and the RONDEL delivery system.

Moving to our obesity program, we announced in July that patient dosing had begun in a phase 1 clinical trial of our drug candidate Adipotide. As you know, that trial is being conducted by investigators at the MD Anderson Cancer Center and is enrolling obese patients with prostate cancer. As the trial is still early and patient recruitment is ongoing, we can't comment about results. However, we have not encountered any treatment related toxicity so far.

We have always viewed the obesity program as a program rather than as a single drug candidate. Our ultimate goal here is not to sell anti-obesity drugs to patients, but rather to eventually license to an appropriate company that is capable of completing clinical development and drug sales and distribution. Therefore, we believe there is more value to our shareholders if we license an entire program, with follow-on and back-up candidates, to a company rather than a single compound. Toward those ends, we have a robust development program that includes internal studies as well as those done in collaboration with Dr. Randy Seeley, Director of the Cincinnati Diabetes and Obesity Center. We are developing new formulations and modified dosing schedules aimed at inducing different levels of weight loss and potentially widened therapeutic windows. We are excited about these developments and expect new publications in peer

reviewed scientific journals in 2013 as well as guidance on possible new clinical candidates and new patient populations.

With the team, tools, and infrastructure now solidly in place, this last quarter has been focused on our three areas of value creation: product development; platform development; and business development. These areas drive everything we do. We are focused on the following priorities over the next 12 months:

1. Present and publish additional data on the advancement of the DPC platform;
2. Push the subcutaneous DPC formulation toward the clinic internally and with partners;
3. Publish non-clinical data on ARC-520;
4. Complete GLP toxicology studies, GMP manufacturing of our clinical supply, and final phase 1 protocol development for ARC-520;
5. File IND or foreign equivalent, for ARC-520;
6. Begin first-in-man studies with ARC-520;
7. Complete library screening and select and deliver peptides in connection with Shire collaboration;
8. Release interim results from the Adipotide phase 1;
9. Publish non-clinical data on additional anti-obesity compounds;
10. Work with Merck on targeting collaboration;
11. Screen targeting peptides and select PDC candidate for internal development;
12. Select additional RNAi candidate for internal development;
13. Sign Homing Peptide and RNAi collaborations and partnerships.

Allow me now to give a brief update on what we know about our licensee Cerulean Pharma's progress on a partnered drug. If you recall, in 2010 we partnered an oncology drug candidate IT-101 (now called CRLX101) and the nanoparticle drug delivery system Cyclosert. This is based on the same cyclodextrin-based polymer technology as RONDEL but optimized to deliver small molecule drugs. Data released on CRLX101 have been positive and Cerulean recently announced that it has completed enrollment of 150 patients in a phase 2 trial in non-small cell lung cancer. This trial has an overall survival endpoint, which is the gold standard for oncology drugs, and a very large non-small cell lung cancer indication. Data from this trial are expected to be released in early 2013. Our license agreement included milestone payments and 10-40% of sub-licensing revenue if Cerulean partners that drug candidate. This will most likely be closer to 10% based on the amount of money we believe they have invested in the development of CRLX101. Cerulean has stated publicly that partnership discussions are under way, and that they plan to start talking about potential deal terms as early as January. With the well-known patent cliff approaching, phase 3 ready assets are in high demand so we are watching this closely and will update shareholders as new information becomes available.

They have also announced that they anticipate another drug candidate to be created with the Cyclosert platform and the chemotherapeutic Docetaxel in early 2013. We are also eligible for milestones payments on that candidate. We applaud our friends at Cerulean for their great work moving CRLX101 and the Cyclosert platform forward. We wish them continued success.

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 earlier today, our net loss attributable to Arrowhead for the year ended September 30, 2012 was $21.1 million, or $1.90 per share based on 11.1 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of $3.1 million, or $0.44 per share based on 7.2 million weighted average shares outstanding, for the year ended September 30, 2011.

For the quarter, our net loss attributable to Arrowhead was $5.3 million, or $0.43 per share based on 12.5 million weighted average shares outstanding. This compares with a net loss of $2.8 million, or $0.39 per share based on 7.2 million weighted average shares outstanding, for the quarter ended September 30, 2011.

On a consolidated basis, for the year ended September 30, 2012, net cash used in operating activities of continuing operations totaled $15.3 million, compared with $7.7 million in the prior-year period.

Our total operating expenses for the year ended September 30, 2012 was $21.2 million, of which approximately $3 million was noncash expenses. Operating expenses in fiscal 2011 were $10.1 million. The increase in operating expenses was primarily related to the acquisition of our Madison R&D facility, and related operating costs, including personnel and research and development activities.

Our total operating expenses for the quarter ended September 30, 2012 were $5.4 million, and increase of $2.5 million from $2.9 million during the quarter ended

September 30, 2011. The increase in operating expenses for the quarter was also due to the Madison R&D facility.

Turning to our balance sheet, our cash position was $3.4 million at September 30, 2012, compared to $7.5 million at September 30, 2011. The change in cash is primarily due to cash used in operations of $15.3 million, somewhat offset by cash inflow from financing activities of $10.8 million.

Our shares outstanding at September 30, 2012, were 13.6 million up 4.9 million from 8.6 million at September 30, 2011. The increase in shares outstanding is primarily due to financings during fiscal 2012.

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

Chris Anzalone

Thank you Ken.

The last twelve months have brought dramatic change to Arrowhead. We have executed on our long-term strategy to transition from being a nanotechnology holding company with independent subsidiaries in multiple industries to a focused and unified biotech company. This transition was not a rebranding effort. Rather, it was a comprehensive rebuilding of our business including our technology platforms, R&D capabilities, and operating, scientific, and business development management. Arrowhead now has a solid foundation to create drugs and

partnerships that will drive long-term value for our shareholders. We are more confident than ever about our ability to do this.

I would like to review some of the key steps that we've taken to make Arrowhead a fundamentally different company than it was just a year ago.

1. Acquired Roche's RNAi business, which included a best in class delivery system in the DPCs, broad siRNA chemistry licenses, a state-of-art 24,000 square foot R&D facility built by big pharma, and R&D staff of approximately 40 scientists.
2. Broadened our management team by adding accomplished biopharma executives Dr. Bruce Given as Chief Operating Officer and Head of R&D, and Dr. Brendan Rae as Chief Business Officer
3. Acquired Alvos Therapeutics providing Arrowhead with a library of peptide targeting sequences used to create peptide-drug conjugates (PDCs) and to serve as targeting agents for Arrowhead's siRNA delivery vehicles
4. Created a centralized infrastructure for the management of clinical trials
5. Dramatically improved our ability to support pharma partnerships and collaborations in RNAi and active-targeting

These steps, along with other accomplishments, have created an integrated and streamlined development operation that allows Arrowhead to advance multiple programs in a short amount of time. We believe our success at taking ARC-520 from an early pre-clinical stage program to an IND-ready asset in just over a year demonstrates this point and positions us as a leader in RNAi drug development. We are proud of the progress we made as a company during the last year, and we believe we are very well positioned to rapidly advance our product candidates and partnering efforts over the next year as well.

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

Operator

Operator opens the call to questions …