

**ARROWHEAD RESEARCH**

**3Q Fiscal 2012 CONFERENCE CALL - PREPARED REMARKS**

**August 13, 2012**

**1:30 PM Pacific time**

**Operator**

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2012 third quarter 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 Mr. Michael Levitan of The Trout Group. Please go ahead Michael.

**Michael Levitan**

Thank you, Operator. Good afternoon, everyone, and thank you for joining us today to discuss Arrowhead's results for its fiscal 2012 third quarter ended June 30, 2011. With us today from management are President and CEO Dr. Christopher Anzalone 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?

<b>Chris Anzalone</b>
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Thanks Michael. Good afternoon everyone and thank you for joining us on our call today. Let me start by mentioning two announcements. First, we will issue a press release before the market opens tomorrow that we have entered into an Evaluation Agreement with Merck, whereby they will assess a novel proprietary antibody against an undisclosed target identified in the human-derived peptide targeting and discovery program, which we acquired in the Alvos transaction. We are very excited about Merck's interest in this program and we believe there will be many

more opportunities to extract value from our portfolio by partnering with biopharma companies in areas such as this. The timing of this is also important. We believe that entering into a collaboration with a company of Merck's stature so soon after acquiring the platform is a tremendous point of validation. We look forward to working with Merck on this and other projects. We also look forward to entering many more collaborations stemming from our targeting library.

The other announcement I would like to touch on is that of our financing. We raised approximately 6.2 million dollars in an offering that was oversubscribed. We believe this was the correct size because it provides us with needed capital while limiting dilution for our existing shareholders. We feel comfortable with our current capitalization given this new financing, the fact that we still have \$14 million in our facility with Lincoln Park Capital, and the stage of development of our technology platforms. Also important in the current financing is the investor group. Approximately half of the funding was provided by current shareholders, led by noted investor Jim Mellon, and the other half was provided by a large healthcare fund. We believe the quality of these investors speaks well of Arrowhead.

Moving to our business, as you know last quarter we acquired Alvos Therapeutics and the platform of homing peptides originally developed at the MD Anderson Cancer Center. I'd like to spend a few moments to talk about what this means for our business and how it has changed the way we position our company to analysts, investors, and potential partners.

The Alvos acquisition completes Arrowhead's transition into a targeted therapeutics company. We are focused entirely on bringing drugs to where they can be effective and nowhere else. The idea of actively targeting drugs is

potentially quite powerful. Guiding drugs to their intended site of action offers the promise of increasing the effectiveness of a drug while also increasing safety by limiting off target effects. Oncology is a prime example of the potential value of targeting. Most oncology drugs have severe side effects that make patients sick and limit how much can be administered. This is largely a targeting problem because cytotoxics are essentially poisons that can kill healthy tissue as well as cancer cells. In fact, it is thought that only a vanishingly small minority of drug actually gets to tumors, while the vast majority goes to healthy tissue. If we can guide those drugs specifically to tumors, we can increase the effectiveness, decrease side-effects, and potentially increase the tolerable dose. The value of this is most obvious in cancer, but similar value could be seen in other indications as well.

This has been a key goal in the pharmaceutical industry for years, and we believe we have something revolutionary in our platform of targeting sequences. Lets review quickly what that is. Phage Display is an established method of discovering novel cell-surface receptors and short peptide sequences that are rapidly internalized by various cell types. This has been used in experimental animals for some time so, while powerful, is not revolutionary. MD Anderson's breakthrough is the use of this technique to generate data directly in humans. Under strict ethical guidelines, they have screened end-stage cancer patients to generate the world's largest library of small peptides that are rapidly taken up by a variety of cells in humans. The result is over 42,000 individual peptide sequences that bind to and are internalized by specific cells. What this means is that each of these 42,000 sequences will enter only a certain cell type and no others. Why should anyone care about this? Because when you link drugs to these peptides, the resulting peptide-drug conjugates (or PDCs) can specifically target the intended tissue. Our library is vast: we have peptides capable of targeting virtually any

tissue in the body. In addition, we can secure rights to new patient screens so the 42,000 sequences could become 100,000 or 200,000. We view this as a powerful land grab that can transform therapeutics across almost any indication.

This targeting platform opens vast new business opportunities for us, and it also supports and enhances the value of our RNAi capabilities. Remember that after our acquisition of Roche's RNAi business, we now have a portfolio of siRNA delivery systems. Our two primary systems, RONDEL and Dynamic Polyconjugates (or DPCs) are both targetable so may be used with our homing peptides. The result is a unified business that leverages multiple platforms to target and deliver a wide range of therapeutic modalities, including small molecule drugs, peptide drugs, and small RNAs. Because our platforms support the development of varied products, we can stay operationally focused while becoming strategically diversified. This enables us to have many shots on goal without falling victim to the challenges of a lack of focus.

We are positioned to address the following four opportunities:

1. Pharmaceutical companies want to make their APIs better. We have over 42,000 targeting peptides capable of homing specifically to virtually any tissue in the body and deliver therapeutic agents to where they can be effective.
2. Pharmaceutical companies want enrichment strategies. Our targeting library can be used to create companion diagnostics to de-risk clinical trials by identifying those patient populations most likely to respond to treatment.
3. We want to make generics better. Taking generics with years of clinical history and targeting them to increase effectiveness and decrease toxicity is a compelling business model. It presents the opportunity to create a

proprietary drug with unique qualities and pricing flexibility without creating a new API.

4. Pharmaceutical companies need reliable ways to deliver siRNA. We have non-lipid targeted delivery platforms that have been validated in rodents, primates, and humans.

We now have multiple targeted therapeutic candidates in development including ARC-520 for treatment of the hepatitis B virus (or “HBV”), Adipotide for obesity, and CALAA-01 for cancer. We also have earlier preclinical oncology programs using generics and RNAi as well as active programs to enable partners to build therapeutics on our platforms.

Since our last conference call we have made important progress on several fronts. Our recent major accomplishments include the following:

1. As I mentioned we acquired the Homing Peptide platform through our Alvos Therapeutics acquisition;
2. Presented data for our DPC siRNA delivery platform and HBV program at six scientific conferences;
3. Began dosing patients in a phase 1 clinical trial of our anti-obesity drug candidate Adipotide;
4. Dosed additional patients in the phase 1b study of CALAA-01;
5. Completed all internal preclinical requirements and selected ARC-520 as our clinical candidate for HBV;
6. Initiated the final IND-enabling steps for ARC-520 including GMP manufacturing and GLP toxicology studies and we recently filed our pre-IND data package with the US FDA;

7. Announced the publication of a new study in the Journal of the American Diabetes Association demonstrating rapid improvement in pro-diabetic markers when obese mice were treated with Adipotide;
8. Strengthened our balance sheet.

Each of these accomplishments represents a critical step toward our goal of developing a product pipeline that can drive long-term value for our shareholders. I'd like to highlight a few of them in more detail.

Last month we announced that patient dosing had begun in a phase 1 clinical trial of Adipotide. The first patient has now completed his one-month treatment. This is a significant step forward and we are eager to review the data as we accrue more patients. The population being studied is obese castrate-resistant prostate cancer patients because long-term hormone therapy leads to obesity in many of these patients. The study is intended to identify a maximum tolerated dose, assess pharmacokinetics, measure the change in weight, and monitor disease progression, in addition to other secondary outcome measures. Since fat tissue is known to produce substances that can promote the growth of some tumors, investigators at MD Anderson, who are bearing all the direct costs of the trial, also want to learn if decreasing white fat can slow the growth of prostate cancer. Although we are not currently testing Adipotide in obese patients without prostate cancer, we believe the safety profile established in this phase 1, should it prove acceptable, will be applicable to a phase 1b or phase 2 trial in a wider obese patient population. The current trial enables us to potentially follow up with one or multiple phase 2s in cancer, obesity, or diabetes.

As you know, Adipotide is a peptide drug that targets a protein on the surface of blood vessels that support white adipose, or fat, tissue. The targeting sequence of

the drug is one of the 42,000 in our library. Once Adipotide is internalized into the cell it induces apoptosis, or programmed cell death, disrupting the blood supply to fat cells and causing them to be metabolized. Further, most of the weight loss observed in rodent and non-human primate studies was due to decreased food intake. This suggests that (a) we are seeing a CNS effect without the drug even entering the CNS; and (b) that animals appear to have a diminished appetite while they are losing weight, which is the opposite of what generally occurs. The former is important from a safety standpoint because we appear to be leveraging the body's own feedback system to decrease appetite rather than directly affecting brain chemistry. The latter is important because the possibility that people may be less hungry while they are losing weight is compelling from a market standpoint.

We also recently announced the publication of a paper in the Journal of the American Diabetes Association which reported that obese mice treated with Adipotide displayed significantly improved insulin sensitivity, improved glucose tolerance, and a reduction in serum triglycerides after only 2-3 days of treatment. These effects occurred independent of and prior to Adipotide-induced weight loss. The research was conducted by a team led by renowned obesity expert Dr. Randy Seeley, Director of the Cincinnati Diabetes and Obesity Center. This study suggests that Adipotide may also be a powerful agent against type II diabetes. This is important because diabetes is the sister epidemic to obesity and it may provide an additional regulatory pathway for Adipotide.

Also during the quarter, Arrowhead scientists presented data at a series of scientific conferences highlighting our DPC siRNA delivery system and our DPC-enabled HBV RNAi therapeutic in development. We believe that our HBV program has the potential to be more effective than current treatments because of its ability to reduce the expression of viral proteins, including HBV Surface Antigen. This is



important because there is currently no cure for HBV and there is a widely held theory that knocking down viral replication *and* viral proteins can lead to a cure in part by enabling the patient's immune system to rebound and clear the rest of the infection. We are very excited about the program and believe that it is uniquely positioned as a potentially powerful new therapeutic. We have seen multi-log reductions in viral protein expression, translating to greater than 99% knockdown in multiple animal models. These results are stunning for their implications to HBV treatment and they demonstrate the power of our targeted DPC delivery system more broadly. In a world where companies get excited about 75 or 80% knockdown, what we have demonstrated is remarkable. We anticipate publication of these results in peer-reviewed scientific journals in the coming months.

In June, we announced that we selected ARC-520 as our HBV clinical candidate. Promoting it as a clinical candidate so soon after acquiring the Roche RNAi assets represents an important milestone for Arrowhead. Having completed all internal preclinical requirements, we initiated the final IND-enabling steps, including GMP manufacturing, GLP toxicology, and we recently filed a pre-IND data package with the US FDA. We have put in place an aggressive development timeline for ARC-520 and anticipate filing an IND, or foreign equivalent, at the end of Q2 2013 and conducting a phase 1 clinical trial in chronic HBV carriers designed to provide early proof of concept. There are thought to be more than 350 million carriers of HBV world-wide and no cure, so needless to say this is an attractive market.

ARC-520 includes two siRNA sequences targeting two different regions of the HBV genome as a strategy to minimize the potential development of resistance in individual patients. The RNA sequences were part of a large-scale screening program initiated by Roche prior to the acquisition by Arrowhead. Consistent with

the company's overarching strategy, the DPCs used in ARC-520 employ active ligand-mediated targeting specific to a receptor on hepatocytes. This approach results in high-potency knockdown of the target gene, validated in mice, rats, and non-human primates and it has demonstrated a low toxicity profile in primates enabling long term dosing.

Since we acquired Alvos in April, we have been working towards the integration of that platform into our existing operations and have begun outreach to potential partners. Initial responses have been positive because we have something that is unique and addresses targeting goals that many companies already have. The Merck collaboration is an important validation of this platform and we are confident that multiple deals will follow.

During the previous 12 months and particularly in the last quarter we worked hard to bring together the necessary resources, expertise, facilities, strategy, and technology platforms to become a unified targeted therapeutics company. Looking towards the future, we are committed to sharpening our focus and making rapid progress in our clinical development programs and executing on our business strategy. Importantly, this includes setting aggressive goals for ourselves and for our product candidates and ensuring that we meet them.

Over the next 12-18 months we expect to reach several important milestones that have the potential to be value-creating events for our shareholders. We will provide updates periodically on our progress towards meeting these milestones, which include the following:

1. Complete the CALAA-01 phase 1b by the end of August, which is just a couple weeks away;

2. Publication of data on our DPC delivery vehicle and our HBV program, anticipated towards the end 2012 through the first half of 2013;
3. Complete IND-enabling steps for ARC-520, including GLP toxicology, GMP manufacturing of our clinical supply, and final phase 1 protocol development – These have various completion times between Q4 2012 and Q2 2013;
4. File IND, or foreign equivalent, for ARC-520 in mid-2013;
5. Release interim results from the Adipotide phase 1;
6. Screen targeting peptides and select PDC candidate for internal development;
7. Select additional RNAi candidate for internal development;
8. Sign Homing Peptide and RNAi collaborations and partnerships.

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?

<b>Ken Myszkowski</b>
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Thank you, Chris, and good afternoon everyone.

As we reported earlier today, our net loss attributable to Arrowhead for the quarter ended June 30, 2012 was \$8 million, or \$0.71 per share based on 11.2 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of \$1.8 million, or \$0.25 per share based on 7.2 million weighted average shares outstanding, for the quarter ended June 30, 2011.

Total operating expenses for the quarter ended June 30, 2012 were \$6.9 million compared with \$1.8 million for the quarter ended June 30, 2011. The increase in

operating expenses includes a \$2 million noncash charge to record a reserve against a receivable from Nanotope. The balance of the increase primarily relates to costs associated with our R&D facility acquired in October of 2011, its associated technical staff, and our research programs.

Net cash used in operating activities for the first nine months of fiscal 2012 was \$10.8 million, compared with \$6.0 million in the prior year period. The increase in cash used in operating activities primarily relates to costs associated with our new R&D facility.

At June 30, 2012, we had cash resources of \$3.3 million, including a cash balance of \$2.3 million, and amounts due from a previous financing of \$1 million. As reported earlier today, we also closed a registered direct offering, gross proceeds will be approximately \$6.2 million prior to commissions and fees.

Our cash at September 30, 2011 was \$7.5 million. Cash outlays for R&D and G&A were \$10.9 million during the first nine months of the fiscal year, and expenditures for capital equipments was \$400,000, while cash inflows consisted of revenues \$200,000, cash from the sale of investments of \$500,000, and proceeds from the sale of equity securities of \$5.4 million. Our shares outstanding at June 30, 2012, were 11.3 million up 2.7 million from 8.6 million at September 30, 2011. The increase in shares outstanding is primarily due shares issued for the Roche Madison acquisition, as well as related financings.

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

Thank you Ken.

The last few months have been important in the obesity and RNAi therapeutics markets.

We believe the recent FDA approvals for Arena and Vivus, the first in 13 years, signal an easing in the regulatory stance and increasing acknowledgement by the FDA that obesity is one of the great healthcare challenges of our times. We see that as a positive signal for Arrowhead, our shareholders, and the millions of patients that struggle with obesity and potentially could benefit from treatment with Adipotide.

On the RNAi front, Alnylam, which is an Arrowhead partner, released some encouraging clinical data on their ALN-TTR02 candidate that sparked a lot of investor interest. The last couple years have been difficult for developers of RNAi therapeutics, but market reaction to the Alnylam data suggest a renewed hunger for good RNAi data, even if it is early. This is encouraging and we believe we are well positioned to benefit from an increased demand, particularly as ARC-520 moves toward the clinic.

During the final few minutes of the call, lets take a look at where we have come strategically and operationally. Less than 10 months ago, we were a company with a single solution for siRNA delivery and a promising pre-clinical obesity program. Both programs were dependent on outsourcing for further development. We are now a fundamentally different company that provides our shareholders with a more

focused business, broader upside potential, increased value to partners, and enhanced ability to rapidly innovate. Through the Roche and Alvos acquisitions, we are now a targeted therapeutics company with deeper value drivers than just 10 months ago. They include:

- A Phase 1 clinical trial in obesity that is fully-funded by MD Anderson
- Completion of a Phase 1b oncology trial in the next 2 weeks
- A very promising program in HBV that will reach the clinic next year
- The world's largest human-derived peptide targeting library that can be used for targeting drugs and the creation of companion diagnostics
- A comprehensive set of RNAi licenses providing broad freedom to operate
- A portfolio of siRNA delivery solutions, including 2 non-lipid platforms that are targetable and validated
- State-of-the-art scientific infrastructure assembled by big pharma that a small biotech company could not afford to build
- Complete experimental animal facilities to rapidly cycle drug development internally; and
- A technical team of over 40 scientists that is second to none

These are some of our tools and we believe that we are focused on the right areas strategically. The pharmaceutical world is increasingly interested in targeting strategies, and we believe that we have the world's most powerful platform to provide this. There is a clear need for siRNA delivery and we believe we have the most powerful platforms to solve this challenge. From a therapeutic standpoint, obesity is garnering tremendous attention, and we are treating patients with a candidate and underlying platform that demonstrated standout preclinical data and a unique mode of action. Hepatitis B remains a large underserved market world

wide that we see as a major focus for the pharmaceutical industry on the heels of tremendous value creation surrounding Hepatitis C. We have a candidate that has demonstrated great promise and will be in the clinic next year. From strategic and operational standpoints, we believe we are on strong footing.

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

<b>Operator</b>
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**Operator opens the call to questions ...**