

ARROWHEAD RESEARCH

1Q Fiscal 2013 CONFERENCE CALL - PREPARED REMARKS

February 13, 2013

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Research fiscal 2013 first quarter and 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 first quarter ended December 31, 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. 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. As we discussed on our last conference call, we viewed 2012 as a transition period for Arrowhead where we sought to comprehensively rebuild our business around the assets, technology, and R&D capabilities acquired through our transaction with Roche. I cannot overstate how dramatically that improved our ability to rapidly advance our product candidates and partnering efforts. In addition, the acquisition of the Homing Peptide platform provided us with a proprietary library of targeting ligands that we believe will broaden our ability to target our siRNA delivery vehicles to various tissues, and potentially allow us to develop a new class of actively targeted drugs in Peptide-Drug Conjugates, or PDCs.

That was 2012. Think of that as a building year and 2013 as a demonstration year. In fiscal and calendar 2013 we are focused on moving our drug candidates forward and publicizing the type of clinical and non-clinical data that represent valuable proof-of-concept. We strive to do this in a capital efficient way, while still providing our scientists with the resources they need to continue to innovate. Our long-term goal is to become less dependent, and ultimately not dependent, on the capital markets by funding our business through partnership, collaboration, and licensing revenues. Generating high-quality clinical data in patients is a critical step toward this, and we believe it starts in 2013. We also see additional significant milestones throughout the year that could serve as catalysts.

Before we go into the milestones targeted for 2013, I would like to briefly review some key accomplishments of the fiscal first quarter and since our last call:

First, we signed a Collaboration and License Agreement with Shire to use our Homing Peptide platform for an undisclosed rare-disease target. The agreement includes research funding, up to \$32.8 million in milestones payments, and royalties on product sales. This is the second agreement we have signed for this platform, the first being with Merck in August of 2012.

Second, we advanced our DPC siRNA delivery platform, which now includes multiple structures optimized for different uses. We presented data in October at the annual meeting of the Oligonucleotide Therapeutics Society in Boston describing a new subcutaneous formulation of DPCs that showed 99% target knockdown in monkeys with no observed toxicity. The efficiency and tolerability of this represents a substantial breakthrough that, we believe, is unmatched in the field. We also published a paper in the journal *Nucleic Acid Therapeutics* that included data demonstrating that

high level target gene knockdown with low doses of cholesterol-conjugated siRNA is possible in non-human primates using DPCs and a novel co-injection strategy. This represents a greater than 500-fold increase in potency compared to cholesterol-conjugated siRNA alone. We are using this strategy and a next generation DPC polymer in ARC-520, our hepatitis B clinical candidate.

Third, we expanded our intellectual property protection through multiple allowed, issued, and filed patent applications. It's important to note that our ARC-520 clinical candidate and other pre-clinical candidates in development that utilize various DPC constructs have patent protection that extends to 2031 and potentially longer. We see the long life of our IP portfolio as an extremely valuable asset.

Fourth, we strengthened our balance sheet through two equity financings for gross proceeds of approximately \$7.5 million. The first financing was completed in December, and because there was excess demand from investors, we extended the offering in January at similar terms. This additional cash extends our runway and allows us to push our development programs to important milestone events, including regulatory filing for ARC-520.

Fifth, we moved ARC-520 toward a planned regulatory filing in the second calendar quarter of 2013 to begin first-in-man studies. We initiated final IND-enabling GLP toxicology studies in December, and the in-life portion of these studies will be complete next week. Thus far the results have been positive and as expected. Histology is due next month and a final audited draft report available in Q2 to support our clinical timeline. We are working

on GMP manufacturing of the clinical supply right now and expect this to be complete in Q2. We recruited a world-class HBV clinical advisory board that is chaired by Dr. Robert Gish. It has assisted the company in the development of our clinical and regulatory strategies.

Looking ahead to our goals in 2013, we see several milestones that have the potential to raise our company's profile and firmly establish Arrowhead as a leader in RNAi therapeutics. As we have seen with Alnylam, investors reward high-quality data with demand for the stock, and pharmaceutical companies reward this validation with demand for licensing and collaboration agreements. Data are impactful when there is wide awareness in the scientific and investment communities, and particularly when data are suggestive of success in the clinic. Throughout 2013 we will release and discuss data through peer-reviewed publications in high impact scientific journals and directly via webcasts, conference calls, and postings on our website. The timing of this activity is important as ARC-520 goes into the clinic this year.

Our goals to communicate our progress include the following:

1. Next month we plan to conduct a webcast describing in detail the development of ARC-520, HBV disease biology, current standard of care, the HBV market opportunity, ARC-520's intended product profile, our clinical trial strategy and timelines, and some exciting new data.
2. Next quarter we will have a webcast on various DPC constructs including hepatocyte targeted DPCs, subcutaneous DPCs, and new tumor targeting structures.
3. Throughout the year we intend to have multiple publications in peer-reviewed scientific journals which will include an HBV paper, a

subcutaneous DPC paper, additional DPC advancements, and papers on peptide targeting.

4. We will also be presenting throughout the year at scientific and investor conferences

As I mentioned, we plan on having a webcast in March about ARC-520, but I wanted to briefly set out the intended timeline and strategy for our first-in-man studies. We are on schedule to file with regulatory authorities in the second calendar quarter of this year. We have decided to conduct two phase 1 studies: a first in man study in healthy volunteers conducted in a Western country, most likely Australia; and a study in chronic HBV infected patients in an Asian country, most likely Hong Kong. We expect to begin treating healthy volunteers in Q3. This study will establish a safety profile relatively rapidly, and may enable us to begin the study in patients at a higher dose, thereby accelerating the path to meaningful results. We are planning the study in patients for Hong Kong because of the high prevalence of HBV, and, therefore, it enables us to recruit patients quickly. Of course we have no control over the time it takes the Hong Kong authorities to approve the study, but we expect to begin treating patients this year. Conducting a phase 1 in HBV patients accomplishes several goals in a short amount of time. Most critically, it allows us to get an early efficacy readout in patients with chronic disease. We hope to be able to measure the drug's ability to knockdown production of new virions as well as viral proteins, including s-antigen, e-antigen, and the core protein that forms the capsid. Knockdown of viral proteins is what many in the field believe will revive the host immune response and potentially provide a functional cure. It is also something that no other current therapy can reliably do, creating an attractive opportunity for us. This trial design is intended to yield a well-rounded data package for a phase 1 asset and has the potential to result in high value creation in a relatively short timeframe. We will

continue to set aggressive goals for clinical data readouts and provide shareholders with updates as we progress.

We also see significant milestones this year for our partner-based programs. Arrowhead licensee Cerulean Pharma currently has several ongoing phase 2 studies of CRLX-101, a polymer-conjugate based on the Cycloset delivery platform and originally designed and developed by Arrowhead scientists, in non-small cell lung cancer, small cell lung cancer, gastric cancer, ovarian cancer, and renal cell carcinoma. The most advanced is a 150 patient non-small cell lung cancer trial, where enrollment has been completed and data is due this quarter. We see the data release as a potential catalyst for us since we retain financial interests in this drug, including a share of sub-licensing revenue, milestones, and royalties on sales. Cerulean has also announced that they anticipate another drug candidate to be created with the Cycloset platform and the chemotherapeutic Docetaxel in early 2013. We are also eligible for milestones payments on that candidate.

Our new partner-based program with Shire is still in its early stages having just signed an agreement with them in December of 2012. We will receive research support for program costs and will also be eligible for milestone payments when we complete the first stage of the collaboration that involves interrogating our Homing Peptide database and delivering peptides to Shire that can target and internalize an undisclosed tissue type.

Moving to our obesity program, patient recruitment is ongoing for 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. We have not yet reached a maximum tolerated dose and do not have enough data to draw any conclusions yet. Patient recruitment

was slowed when the original principal investigator left MD Anderson to join a different institution. A new investigator is now running the trial so we are looking forward to continuing progress. We intend to have some interim results late in the year.

Lastly, we are working on new RNAi drug candidates based on the DPC delivery system. We have not yet announced the disease areas or targets, but we are planning on discussing that later in the year. Since this is still rather early, our timelines are uncertain, however our current goal is to have a designated candidate announced this year and IND-ready next year.

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, 2012 was \$4.6 million, or \$0.33 per share based on 14.1 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of \$2.5 million, or \$0.25 per share based on 10.1 million weighted average shares outstanding, for the three months ended December 31, 2011.

During the three months ended December 31, 2012, net cash used in operating activities was \$3.8 million, compared to \$2.7 million during the prior year quarter.

Total operating expenses for the three months ended December 31, 2012 were \$5.0 million, compared to 4.1 million for the three months ended December 31, 2011.

Operating expenses and cash used in operations were up slightly during the quarter representing a full three months of activity for our Madison operations, as well as higher R&D expenses as we advance ARC-520 through preclinical toxicology testing and related manufacturing costs.

Turning to our balance sheet, our cash position was \$2.9 million at December 31, 2012, compared to \$3.4 million at September 30, 2012. During the quarter, the cash decrease of \$0.5 million was due to operating cash flow of \$3.8 million offset by cash flow from financing activities was \$3.3 million. During January an additional financing yielded net proceeds of \$3.3 million.

Our current shares outstanding are 17.3 million, and were 15.6 million at December 31, 2012. This compares to 13.6 million shares outstanding at September 30, 2012. The increase in shares outstanding is primarily due to the recent financings we completed in December and January.

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

Chris Anzalone

Thank you Ken.

As I mentioned, fiscal 2012 was a building year. We acquired a tremendous set of RNAi assets that Roche invested over 500 million dollars to establish. Later, we

acquired what we believe to be the world's largest human-derived peptide targeting library to use with our siRNA delivery system. These are powerful platforms and we are now in a position to rapidly build products on them. This means converting potential value into actual value and we believe 2013 will represent a tangible leap in that direction. As I mentioned earlier, think of 2013 as a demonstration year. Beginning this quarter, we will have publications, webcasts, and website posts presenting to the world data that demonstrate the power of our platforms and individual drug candidates. This will be the first time much of these data will have been presented outside the company. We expect to enter the clinic with what we believe to be a potential breakthrough hepatitis B treatment. In addition to treating healthy volunteers, we will treat chronic HBV carriers, providing the type of efficacy readout that usually comes with a Phase 2. We plan to continue clinical studies in our obesity program. We are building new clinical candidates and will provide guidance around them and underlying data. In short, we believe that the hard work of 2012 starts to bear substantial fruit this year and provides sustainable long-term value creation. 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 ...