October 14, 2015

Arrowhead Presents Overview of Its Broad RNAi Delivery Platform and Introduces New Subcutaneously Administered Format

PASADENA, Calif.--(BUSINESS WIRE)--

Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today presented an overview of its broad RNAi delivery platform at the Annual Meeting of the Oligonucleotide Therapeutics Society in Leiden, the Netherlands. Included in this presentation was data from a new program, ARC-LPA, which is designed to reduce production of apolipoprotein A [apo(a)], a key component of lipoprotein(a) [Lp(a)]. Lp(a) has been genetically linked with increased risk of cardiovascular diseases, independent of cholesterol and LDL levels. Using a new subcutaneous delivery construct that Arrowhead has developed, an initial lead compound has achieved up to 90% knockdown of apo(a) in mice.

"Our ability to generate RNAi drugs for a wide variety of indications has expanded dramatically with our advances in delivery," said Chris Anzalone, Ph.D., Arrowhead's president and chief executive officer. "We currently have two drugs in the clinic, ARC-520 and ARC-AAT, that validate our capabilities in IV liver delivery and provide additional confidence in follow-on candidates ARC-F12 and ARC-521. We are now able to deliver to tumors, as exemplified by ARC-HIF2, and report data today on subcutaneous administration for liver delivery with ARC-LPA. These capabilities provide broad opportunities to fight diverse diseases and enable us to drive an aggressive pipeline."

In a presentation titled, "**Development of RNAi-Based Therapeutics using Dynamic Polyconjugates™ (DPC™) Technology**," Bruce Given, M.D., Arrowhead's chief operating officer, discussed three examples of Arrowhead's broad RNAi delivery platform: DPC™ for liver delivery with select data from the ARC-520 clinical program; DPC™ for extra-hepatic delivery with select preclinical data from the ARC-HIF2 program; and, a new proprietary subcutaneous delivery vehicle with initial preclinical data from the ARC-LPA program.

Arrowhead uses DPC™, or Dynamic Polyconjugates™, for liver delivery in both of its clinical stage drugs, ARC-520 for chronic hepatitis B infection and ARC-AAT for alpha-1 antitrypsin deficiency, as well as additional preclinical stage programs, including ARC-F12 for hereditary angioedema and ARC-521 for chronic hepatitis B infection. Arrowhead's delivery platform can produce deep and sustained knockdown of mRNA and proteins, as evidenced by data from the ARC-520 program showing dramatic reductions in hepatitis B e-antigen, core-related antigen, and s-antigen in humans. ARC-520 achieved a maximum reduction of s-antigen of 99% (1.9 log), which is the highest reported single-dose knockdown in humans with any RNAi therapeutic.

DPC™ for extra-hepatic delivery is being deployed in Arrowhead's ARC-HIF2 candidate for the treatment of clear cell renal cell carcinoma (ccRCC). As Arrowhead previously reported, in an orthotopic ccRCC tumor model in mice ARC-HIF2 reduced the expression of HIF2α, a well-validated oncogene in this disease, by more than 80%, leading to statistically significant reductions in tumor size and weight, extensive tumor cell death, reduction in the tumor-expressed VEGF-A biomarker, and destruction of the blood vessels feeding the tumors. DPC™ for extra-hepatic delivery employs a masked polymer designed to induce endosomal escape, with RNAi trigger attached directly to the delivery vehicle. This molecule is actively guided to tumor cells with a proprietary targeting agent that binds to αVβ3 integrin, which is highly expressed on the surface of ccRCC cells as well as various other tumor types.

Dr. Given also introduced Arrowhead's new hepatic delivery format being developed for subcutaneous administration and a new target being explored for cardiovascular disease. The new delivery construct discussed includes an RNAi trigger against apo(a). Apo(a) is a critical component of Lp(a) and effective knockdown results in marked Lp(a) reductions. Single dose knockdown of around 90% in apo(a) has been achieved in mice. Initial data through day 15 suggest a good duration of effect (study ongoing). Similar to the intravenously administered vehicle used for hepatic delivery, this construct employs N-acetyl galactosamine for hepatic targeting. However, it does not employ the same peptide-based active endosomal escape. An active lead compound has been identified and additional lead optimization is ongoing. This program has not been officially designated as entering pre-IND development.

A copy of the presentation can be accessed by visiting the Events section of the company's website at http://ir.arrowheadresearch.com/events.cfm after the presentation concludes.

About Arrowhead Research Corporation

Arrowhead Research Corporation is a biopharmaceutical company developing targeted RNAi therapeutics. The company is leveraging its proprietary Dynamic Polyconjugate™ delivery platform to develop targeted drugs based on the RNA interference mechanism that efficiently silences disease-causing genes. Arrowhead's pipeline includes ARC-520 and ARC-521 for chronic hepatitis B virus, ARC-AAT for liver disease associated with Alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic diseases, and ARC-HIF2 for renal cell carcinoma.

For more information please visit http://www.arrowheadresearch.com, or follow us on Twitter @ArrowRes. To be added to the Company's email list and receive news directly, please visit

http://ir.arrowheadresearch.com/alerts.cfm.

Safe Harbor Statement under the Private Securities Litigation Reform Act:

This news release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These statements are based upon our current expectations and speak only as of the date hereof. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of various factors and uncertainties, including our ability to finance our operations, the future success of our scientific studies, our ability to successfully develop drug candidates, the timing for starting and completing clinical trials, rapid technological change in our markets, and the enforcement of our intellectual property rights. Arrowhead Research Corporation's most recent Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q discuss some of the important risk factors that may affect our business, results of operations and financial condition. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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