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Arrowhead Nominates ARC-HIF2 As First RNAi Therapeutic Candidate Using Extrahepatic DPC™ Delivery System

Data to be presented September 27 at the European Cancer Conference 2015 in Vienna

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that it has nominated ARC-HIF2 as its first therapeutic candidate delivered using a new Dynamic Polyconjugate™ (DPC™) designed to target tissues outside of the liver. Arrowhead believes that ARC-HIF2, which uses RNA interference to silence transcription factor hypoxia-inducible factor 2α (HIF-2α), is a promising new candidate for the treatment of clear cell renal cell carcinoma (ccRCC). The company will present preclinical data at the European Cancer Congress 2015 (ECC2015) in Vienna on September 27 in a session starting at 16:45 CEST. In a poster titled "HIF-2α targeting with a novel RNAi delivery platform as therapy for renal cell carcinoma," (abstract #353), Arrowhead scientists will show data suggesting that HIF-2α inhibition through RNA interference may significantly impact late stage ccRCC progression. The company is in the process of manufacturing scale up to allow for initiation of IND-enabling studies. Timing for anticipated regulatory submission will be announced in the future.

"Preclinical data using our new extrahepatic DPC™ delivery system has been very promising. We think the ability to target tissues outside of the liver, including tumors, opens additional opportunities for Arrowhead to develop differentiated RNAi-therapeutics that address numerous diseases without adequate treatment options," said Christopher Anzalone, Ph.D., Arrowhead's president and chief executive officer. "This is an important milestone for Arrowhead and we look forward to continued development of the DPC™ delivery platform and product candidates based on it."

ARC-HIF2 is designed to inhibit the production of HIF-2α, which has been linked to tumor progression and metastasis in ccRCC. ARC-HIF2 employs a novel extrahepatic-targeted DPC™ that comprises a membrane active polymer to promote RNAi trigger endosomal release, an active ligand that targets the DPC™ to tumor cells, reversible masking to prevent polymer activity prior to cellular uptake, and an RNAi trigger to HIF-2α conjugated directly to the DPC™.

Using ARC-HIF2 in a preclinical ccRCC tumor model, mice treated with weekly injections led to greater than 80% knockdown of HIF-2α mRNA in tumors. Furthermore, tumors from treated mice exhibited statistically significant reductions in size and weight, extensive tumor cell death, reduction in the tumor-expressed VEGF-A biomarker, and destruction of the blood vessels feeding the tumors.

Therapies for metastatic ccRCC including agents that target the VEGF/VEGFR or mTor signaling pathways, which are validated cancer targets, have become the standard-of-care and have improved patient outcomes. However, since emergence of resistance to these agents is common, novel therapies targeting alternative pathways are needed for patients with resistant tumors. Arrowhead believes that HIF2α is an attractive target for intervention because over 90% of ccRCC tumors express a mutant form of the Von Hippel-Landau protein that is unable to degrade HIF-2α, leading to its accumulation during tumor hypoxia and promoting tumor growth.

An abstract of the data to be presented at ECC2015 is available on the conference website at www.europeancancercongress.org/.

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 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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