April 30, 2015

Arrowhead Publishes Data on Advances in Subcutaneous siRNA Delivery

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced the publication of new data on a subcutaneously administered formulation of its Dynamic Polyconjugate (DPC) delivery system. The company believes the new DPCs are highly potent and may represent a dramatic improvement in duration of activity over competing technologies. This new class of DPCs may also enable targeting of RNAi therapeutics to tissues outside of the liver. The manuscript entitled, "Protease-triggered siRNA delivery vehicles," by David B. Rozema et al, was made available online ahead of print in the Journal of Controlled Release 209 (2015) 57-66.

In the publication, Arrowhead scientists describe the development of protease-sensitive masking chemistries that are used in a class DPCs where the RNAi trigger molecule is conjugated directly to the polymer delivery vehicle. These new vehicles expand on the company's existing acid labile DPCs, where the polymer and RNAi trigger are two separate molecules and are co-injected. The protease-sensitive linkages appear to be more stable and have longer circulation times, which may allow for an increased range of targeting, and appear amenable to subcutaneous administration. The publication reports a high level of target gene knockdown and long duration of effect can be achieved after subcutaneous injection with these new DPC delivery vehicles in nonhuman primates. After a single subcutaneous injection of 0.5 mg/kg siRNA against Factor 7 (FVII), a liver expressed coagulation factor that is secreted into the bloodstream, a 99% reduction of FVII activity was observed in nonhuman primates. Further, this reduction was highly durable with maximal knockdown occurring 24 days after injection and measurable reduction in FVII activity appears to persist for up to 200 days.

"This publication speaks to the flexibility of the DPCs and the ability of our scientists to continually expand the platform to enable additional uses," said Christopher Anzalone, Ph.D., president and chief executive officer. "These data suggest that subcutaneously administered DPCs that employ the protease sensitive masking chemistries may be well suited to address chronic diseases of the liver that require long-term treatment and where long dosing intervals would be very attractive to physicians and patients."

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 and ARC-AAT for liver disease associated with Alpha-1 antitrypsin deficiency.

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, actions of the U.S. Food and Drug Administration (FDA) and similar global regulatory bodies, rapid technological change in our markets, challenges to the validity of our intellectual property rights, 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.

Source: Arrowhead Research Corporation

Arrowhead Research Corporation
Vince Anzalone, CFA
626-304-3400
ir@arrowres.com
or
Investor Relations:
The Trout Group
Todd James
646-378-2926
ir@arrowres.com
or
Media:
Russo Partners
Matt Middleman, M.D.
212-845-4272
matt.middleman@russopartnersllc.com

Source: Arrowhead Research Corporation

News Provided by Acquire Media