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Arrowhead Pharmaceuticals' Preclinical Candidate ARC-LPA Achieves 98% Knockdown and Long Duration of Effect after Subcutaneous Administration

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today presented a poster on ARC-LPA, its preclinical development program targeting lipoprotein (a), or Lp(a), for the treatment of cardiovascular disease at the Arteriosclerosis, Thrombosis and Vascular Biology | Peripheral Vascular Disease (ATVB|PVD) 2016 Scientific Sessions in Nashville. ARC-LPA is the first RNAi therapeutic program to use Arrowhead's new delivery vehicles designed for subcutaneous (SQ) administration.

Chris Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, said: "These data are exciting for several reasons. First, they represent continued progress in our SQ delivery platform, creating opportunities to address various diseases that require chronic treatment and where SQ administration may be preferable for patients and physicians. We are enabling very deep target gene knockdown with long duration of effect that may enable monthly, bi-monthly, or even less frequent administration. Second, reducing Lp(a), as we have seen now in multiple animal models, could represent an important leap forward in the treatment of cardiovascular disease. High levels of Lp(a) are associated with increased risk of cardiovascular disease independent of cholesterol and LDL, and there is currently no good way to deeply reduce circulating levels of Lp(a)."

The poster titled, "Lipoprotein(a) targeting with RNAi delivery platforms in transgenic mice and cynomolgus monkeys" (presentation 428), describes data from in vitro screening of RNAi triggers and multiple in vivo models, including transiently transgenic mice, transgenic mice (Tg), and non-human primates (NHPs). Key findings from these studies include the following:

- Screening of RNAi triggers in Tg mice identified those that exhibited substantial and sustained knockdown of serum apo(a) and Lp(a) levels
- RNAi trigger sequences were active in both intravenous and SQ platforms
- Structure activity relationship (SAR) studies looking at chemical modifications to the RNAi trigger identified a lead that demonstrated greater than 98% maximum knockdown after a single 3 mg/kg SQ dose in transgenic mice
- Duration of effect gains were also made with greater than 85% knockdown still seen at 6 weeks post dose
- In NHPs, 85-90% reduction of serum Lp(a) levels was observed after three weekly 3 mg/kg SQ doses
- Duration of effect in NHPs was long, with Lp(a) levels still reduced by 75% 6 weeks after the final dose

A copy of the poster presentation will be made available on the Events and Presentations page under the Investors section of the Arrowhead website.

About ARC-LPA

Arrowhead's RNAi-based candidate ARC-LPA is in preclinical development as a potential treatment for cardiovascular diseases. ARC-LPA is designed to reduce production of apolipoprotein(a), a key component of lipoprotein(a), or Lp(a). Lp (a) levels in humans are genetically defined and higher levels correlate with increased risk of cardiovascular diseases, independent of cholesterol and LDL levels. ARC-LPA is Arrowhead's first drug candidate to use a subcutaneously administered delivery construct.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, Arrowhead therapies trigger the RNA interference mechanism to induce rapid, deep, and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead's RNAi-based therapeutics leverage this natural pathway of gene silencing. The company's pipeline includes ARC-520 and ARC-521 for chronic hepatitis B virus infection, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic disorders, ARC-LPA for cardiovascular disease, and ARC-HIF2 for renal cell carcinoma.

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

http://ir.arrowheadpharma.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. Our 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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