Arrowhead Pharmaceuticals Presents Preclinical Data on Expanding Cardiometabolic Pipeline

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PASADENA, Calif.--(BUSINESS WIRE)--May 11, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced the presentation of new preclinical data on its expanding pipeline of RNA interference (RNAi) therapeutics for cardiometabolic diseases, including ARO-ANG3, which targets angiopoietin-like protein 3 (ANGPTL3), and ARO-APOC3, which targets apolipoprotein C-III (ApoC3). The invited lecture, titled “The promise of RNA interference as a therapeutic approach for treatment of cardiovascular diseases,” was presented at the Vascular Discovery: From Genes to Medicine – Scientific Sessions 2018, a symposium organized by the American Heart Association. Arrowhead intends to file clinical trial applications (CTA) for ARO-ANG3 and ARO-APOC3 before the end of 2018.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead, said, “We have a growing body of compelling preclinical data in a diverse set of models for ARO-APOC3 and ARO-ANG3, the newest candidates in Arrowhead’s cardiometabolic pipeline that leverage our Targeted RNAi Molecule (TRiM^{TM}) platform. We intend to present additional results from ongoing preclinical studies of ARO-APOC3 and ARO-ANG3 at scientific conferences later this year. We also look forward to filing CTAs late this year to advance these promising product candidates into the clinic.”

In the presentation, Dr. Given discussed the recent progress achieved using RNA interference as a therapeutic modality for the development of new drugs to treat cardiovascular diseases. The new preclinical data presented on ARO-APOC3 and ARO-ANG3 included the following::

- A single 2 mg/kg dose of ARO-APOC3 in ApoC3 transgenic mice led to the following observations:
  - Serum ApoC3 levels were reduced by approximately 90%
  - Maximum knockdown was sustained for over three weeks
  - The lipid profiles improved, with large reductions in serum triglycerides and cholesterol, and large increases in HDL
- In dose ranging studies of ARO-ANG3 in wild type mice, reductions in serum ANGPTL3 and liver mRNA were similar, indicating that the observed knockdown of serum ANGPTL3 is a direct result of the expected RNAi effect.
- The following mouse disease models were also interrogated:
  - LDL receptor knock-out (LDLr$^{-/-}$) mice
  - Leptin receptor defective db/db mice
  - Diet-induced obese (DIO) mice
- A single 3 mg/kg dose in LDLr$^{-/-}$ mice and in db/db mice led to:
  - A maximum reduction of ANGPTL3 of 98%
  - Substantial decreases in serum triglycerides and LDL
- A single 3 mg/kg dose in DIO mice led to:
  - A maximum reduction of ANGPTL3 of 99.7%
  - Approximately 50-60% reductions in serum triglycerides and LDL

A copy of presentation materials may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

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.

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/email-alerts.

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 the safety and efficacy of our product candidates, the duration and impact of regulatory delays in our clinical programs, 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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