Arrowhead Reports Interim Clinical Data on Cardiometabolic Candidates ARO-APOC3 and ARO-ANG3

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- Multiple-dose data across various patient populations show marked improvement in multiple lipid parameters

- Company targets initiation of pivotal studies in 2020

PASADENA, Calif.--(BUSINESS WIRE)--Feb. 5, 2020-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today reported promising interim clinical results from the ongoing Phase 1/2a studies of its two RNAi-based cardiometabolic candidates, ARO-APOC3 targeting apolipoprotein C-III (APOC3) being developed as a potential treatment for patients with severe hypertriglyceridemia, and ARO-ANG3 targeting angiopoietin like protein 3 (ANGPTL3) being developed as a potential treatment for dyslipidemias and metabolic diseases.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: “The interim results from the multiple-dose portion of the Phase 1 studies of Arrowhead’s cardiometabolic candidates ARO-APOC3 and ARO-ANG3 are highly encouraging and support our belief that RNAi may be the optimal mechanism to inhibit APOC3 and ANGPTL3. We achieved high levels of APOC3 and ANGPTL3 protein knockdown, which led to impressive reductions in triglycerides and other lipid parameters. In addition, the long duration of effect of ARO-APOC3 and ARO-ANG3 enables a convenient once every 4 months or, possibly, once every 6 months dosing regimen, which also has the potential to improve patient compliance over other agents and mechanisms that require more frequent dosing. From a safety and tolerability perspective, both ARO-ANG3 and ARO-APOC3 data continue to look similar to our other TRiM ™ candidates. There have been no drug-related discontinuations, and the most common adverse events reported were headache, respiratory tract infections, and local injection site reactions. This high level of pharmacologic activity with good safety and tolerability to date is precisely what we were hoping for. We look forward to further investigating the potential for ARO-APOC3 and ARO-ANG3 to provide clinical benefits in patients.”

Key interim results from the multiple-dose portion of the AROAPOC31001 Phase 1 clinical study of ARO-APOC3 include the following:

- Severe hypertriglyceridemia patients with at least 29 days of data after receiving the first dose (50 mg dose, n=3)
  - Mean maximum reductions in APOC3 of 97%
  - Mean maximum reductions in triglycerides (TG) of 95%
  - Mean maximum absolute reduction in TG of -3183 mg/dL

Key interim results from the multiple-dose portion of the AROANG1001 Phase 1 clinical study of ARO-ANG3 include the following:

- Hypercholesterolemia patients on a stable LDL-C lowering treatment regimen including statins, with or without ezetimibe, and some receiving PCSK9 inhibitors, with at least 29 days of data after receiving the first dose (100-300 mg dose, n=22)
  - Mean maximum reductions in ANGPTL3 of 79-88%
  - Mean maximum reductions in LDL-C of 39-42%

- Hypertriglyceridemia patients with at least 29 days of data after receiving the first dose (200 mg dose, n=5)
  - Mean maximum reductions in ANGPTL3 of 83%
  - Mean maximum reductions in TG of 79%

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 likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize 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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