Arrowhead Presents New Clinical Data on ARO-APOC3 at AHA 2021

November 15, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 15, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced additional Phase 1/2 clinical data on ARO-APOC3, the company's investigational RNA interference (RNAi) therapeutic targeting apolipoprotein C-III (APOC3) being developed as a treatment for patients with hypertriglyceridemia (HTG), severe hypertriglyceridemia (sHTG), and familial chylomicronemia syndrome (FCS), at the American Heart Association (AHA) Scientific Sessions 2021.

Four genetically confirmed FCS patients received 50 mg ARO-APOC3 and 26 multifactorial chylomicronemia (MCM or non-FCS) patients received 10, 25, 50, or 100 mg ARO-APOC3 on days 1 and 29. Since similar responses were observed among MCM pts, results were pooled across dose levels. Safety and pharmacodynamic responses were examined. Maximal mean and median changes from baseline for APOC3, triglycerides (TG), non-HDL-C, and HDL-C were reported.

Key data presented include the following:

Summary

- In patients with FCS compared with non-FCS, ARO-APOC3 achieved similar levels of reduction of APOC3 and changes in key lipid parameters
- In patients with FCS compared with non-FCS, safety parameters were similar and comparable
- In patients with sHTG, ARO-APOC3 was well tolerated, and consistently decreased APOC3, TG, and non-HDL-C, and increased HDL-C, independent of underlying genetic cause of HTG
- ARO-APOC3 may represent a promising RNAi therapeutic for the treatment of sHTG with infrequent dosing of every 3 months or every 6 months

Pharmacodynamic Response

- APOC3 was reduced by 98% in FCS patients and 96% in MCM patients
- Both groups showed similar maximum median reductions in TG of 91% and 90%, respectively
- Non-HDL-C was reduced by 58% and 49%, respectively
- HDL-C increased by 152% and 111%, respectively

Safety

- ARO-APOC3 was generally well tolerated
- No treatment emergent adverse event (TEAE)-related study drug discontinuation, dose interruptions, or premature study withdrawals
- No clear pattern of an increased frequency or intensity of adverse events with increasing dose level
- TEAEs and the safety parameters were similar and comparable with FCS compared to non-FCS subjects
- 2 serious adverse events (chest pain and acute pancreatitis) not related to ARO-APOC3 in 2 subjects in the non-FCS group – both subjects completed the study

A copy of the 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. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "hope," "intend," "plan," "project," "could," "estimate," or "continue" are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects or benefits of our collaborations with other companies, or other characterizations of future events or circumstances are forward-looking statements. 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 numerous factors and uncertainties, including the impact of the ongoing COVID-19 pandemic on our business, the safety and efficacy of our product candidates, decisions of regulatory authorities and the timing thereof, 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, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K,

subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

Source: Arrowhead Pharmaceuticals, Inc.

View source version on businesswire.com: https://www.businesswire.com/news/home/20211115005284/en/

Arrowhead Pharmaceuticals, Inc.
Vince Anzalone, CFA
626-304-3400
ir@arrowheadpharma.com

Investors:
LifeSci Advisors, LLC
Brian Ritchie
212-915-2578
britchie@lifesciadvisors.com
www.lifesciadvisors.com

Media:
LifeSci Communications, LLC
Josephine Belluardo, Ph.D.
646-751-4361
jo@lifescicomms.com
www.lifescicommunications.com

Source: Arrowhead Pharmaceuticals, Inc.