September 14, 2017

Arrowhead Hosts Investor & Analyst R&D Day to Introduce TRiM™ Platform and Lead RNAi-based Drug Candidates

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR), is hosting an investor & analyst R&D day in New York today to introduce its proprietary Targeted RNAi Molecule (TRiM™) platform and review its pipeline of RNAi therapeutic candidates.

Chris Anzalone, Ph.D., president and chief executive officer of Arrowhead Pharmaceuticals, said: "We are excited to introduce our new TRiM™ platform and the first development candidates to be advanced: ARO-HBV, targeting chronic hepatitis B infection; ARO-AAT, targeting alpha-1 antitrypsin deficiency liver disease; and, ARO-APOC3 and our newest candidate ARO-ANG3, targeting hypertriglyceridemia. TRiM™ is characterized by its ligand-mediated delivery strategy, structural simplicity, and trigger selection technology, which is an important part of TRiM™ that enables us to rapidly develop what we believe are optimal RNAi therapies. It also allows tissue-specific targeting and to that end we are announcing today that we have seen some very exciting results using TRiM™ technology to target genes and diseases in the lung. This opens up a host of new opportunities for disease targets previously not accessible to RNAi therapeutics and Arrowhead is thrilled to be leading the industry in these efforts."

The R&D day will feature presentations by key opinion leaders, Jeffrey Teckman, M.D. (St. Louis University School of Medicine), Stephen Locarnini, M.D., Ph.D. (Victorian Infectious Diseases Reference Laboratory), and Ira Goldberg, M.D. (NYU Langone Medical Center), as well as Arrowhead management.

A live and archived webcast of the event can be accessed on the Events and Presentations page on the Investors section of the Arrowhead website at http://ir.arrowheadpharma.com/events.cfm.

Agenda and Approximate Times for Discussion Topics

12:00-12:45 Lunch
1:00-1:05 Welcome and Introductions - Vince Anzalone, CFA
1:05-1:35 TRiM™ Platform and Pipeline Review - Chris Anzalone, Ph.D.
1:35-1:55 Hepatitis B - Stephen Locarnini, M.D., Ph.D.
1:55-2:05 ARO-HBV - Bruce Given, M.D.
2:05-2:25 Alpha-1 Antitrypsin Deficiency Liver Disease - Jeffrey Teckman, M.D.
2:25-2:35 ARO-AAT - Bruce Given, M.D.
2:35-2:55 Hypertriglyceridemia - Ira Goldberg, M.D.
2:55-3:05 ARO-APOC3 and ARO-ANG3 - Bruce Given, M.D.
3:05-3:15 Concluding Remarks - Chris Anzalone, Ph.D.
3:15-3:35 Q & A - Panel

Select R&D Day Highlights

Targeted RNAi Molecule Platform (TRiM™)

Arrowhead's Targeted RNAi Molecule platform, or TRiM™, utilizes ligand-mediated delivery and is designed to enable multi-tissue targeting, while being structurally simple. Active targeting has been core to Arrowhead's development strategy and the TRiM™ platform builds on more than a decade of work on actively targeted drug delivery vehicles. Arrowhead scientists now have the ability to progressively "TRiM" away extraneous features and chemistries and retain optimal pharmacologic activity.

New drug candidates utilizing this technology can achieve high levels of target gene knockdown, without an active endosomal escape component, as was required with prior technology platforms.

The TRiM™ platform represents an evolution in RNAi therapeutics from biologic complexity to small molecule precision and

execution. The TRiM™ platform comprises the following components optimized, as needed, for each drug candidate: high affinity targeting ligands; various linker chemistries; structures that enhance pharmacokinetics; and highly potent RNAi triggers with sequence specific stabilization chemistries. The platform offers several competitive advantages, including:

- Simplified manufacturing at reduced cost
- Multiple routes of administration (subcutaneous, intravenous, and inhaled)
- Faster time to clinical candidates
- Wide safety margins
- Promise of taking RNAi to tissues beyond the liver

Extra-Hepatic Targeting

Arrowhead believes that for RNAi to reach its true potential, it must target organs outside the liver. Arrowhead is leading this expansion with the TRiM™ platform that holds the promise of reaching multiple tissues, including the lung and tumors. ARO-Lung1, the first candidate against an undisclosed gene target in the lung, reached almost 90% target knockdown following inhaled administration in rodents. In addition, the ARO-HIF2 candidate targeting renal cell carcinoma achieved 85% target gene knockdown in a rodent tumor model. Clinical Trial Authorization (CTA) filings are planned in Q4 2018 and in 2019 for ARO-Lung1 and ARO-HIF2, respectively.

ARO-AAT

ARO-AAT, Arrowhead's second generation subcutaneously administered clinical candidate for the treatment of alpha-1 antitrypsin deficiency liver disease, achieved up to 92% knockdown in monkeys, thought to be near complete suppression of hepatic production of the alpha-1 antitrypsin protein. In non-GLP rat and monkey exploratory toxicology studies, no changes in clinical chemistries were observed and no histopathology suggestive of organ toxicity at doses up to 300 mg/kg (100x expected human dose). Arrowhead plans to file a CTA in Q1 2018 to initiate clinical studies, pending completion of GLP toxicology studies.

ARO-HBV

ARO-HBV, Arrowhead's third generation subcutaneously administered clinical candidate for the treatment of chronic hepatitis B virus infection, achieved up to 99.9% knockdown of hepatitis B surface antigen (HBsAg), e-antigen (HBeAg), and HBV DNA in rodent models. In a non-GLP rat exploratory toxicology study, no changes in clinical chemistries or histopathology changes suggestive of organ toxicity were observed at doses up to 300 mg/kg (75-100x expected human dose). Arrowhead plans to file a CTA in Q2 2018 to initiate clinical studies, pending completion of cross-reactivity and GLP toxicology studies.

ARO-APOC3 and ARO-ANG3

Arrowhead is expanding its cardiovascular disease portfolio utilizing the TRiM™ platform. Added to existing programs, ARO-LPA and ARO-AMG1, both partnered with Amgen; will be ARO-APOC3, targeting apolipoprotein C-III, and ARO-ANG3, targeting angiopoietin-like protein 3 (ANGPTL3). ARO-APOC3 and ARO-ANG3 will both be developed for the treatment of hypertriglyceridemia. CTA filings are planned for one or both candidates by the end of 2018.

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/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 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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