



Arrowhead Completes Enrollment in Phase 1 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

June 18, 2018

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 18, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has completed enrollment of a Phase 1 clinical study of ARO-AAT, the company's second generation subcutaneously administered RNA interference (RNAi) therapeutic being developed as a treatment for a rare genetic liver disease associated with alpha-1 antitrypsin deficiency. Two planned cohorts at a dose of 400 mg were eliminated because maximal activity appeared to occur at lower doses than expected. The company intends to submit a late-breaking abstract with initial clinical data on ARO-AAT to the Liver Meeting[®], the Annual Meeting of the American Association for the Study of Liver Disease (AASLD), being held in November 2018.

Chris Anzalone, Ph.D., Arrowhead's president and chief executive officer, said, "The ARO-AAT Phase 1 study provides the first readout on pharmacologic activity for a therapeutic leveraging our proprietary Targeted RNAi Molecule, or TRiM[™], platform. We have escalated above a dose that we believe achieves maximal activity, and all doses to date appear to be generally well-tolerated."

AROAT1001 ([NCT03362242](https://clinicaltrials.gov/ct2/show/study/NCT03362242)) is a Phase 1 single- and multiple-ascending dose study to evaluate the safety, tolerability, pharmacokinetics, and effect of ARO-AAT on serum alpha-1 antitrypsin levels in healthy adult volunteers. The study includes 7 cohorts in which 16 subjects receive placebo and 28 subjects receive single or multiple doses of ARO-AAT at doses of 35, 100, 200, or 300 mg. Additional cohorts were planned at a dose of 400 mg, but were deemed unnecessary based on observed activity at lower doses.

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](https://twitter.com/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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