



December 20, 2017

Arrowhead Pharmaceuticals Files for Regulatory Clearance to Begin Phase 1 Study of ARO-AAT

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it filed a regulatory submission to begin a first-in-human study of ARO-AAT, a second-generation investigational medicine for the treatment of alpha-1 liver disease that leverages Arrowhead's subcutaneously administered Targeted RNAi Molecule (TRiM™) technology. Pending approval, Arrowhead intends to proceed with AROAAT1001, 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.

Chris Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, said: "ARO-AAT is our second candidate targeting alpha-1 liver disease and represents the first of five planned regulatory submissions over the next twelve months for product candidates built on our TRiM™ platform. We have moved very rapidly with this program and are thrilled to now submit the regulatory filing ahead of schedule."

The application for approval of the clinical trial was submitted to the New Zealand Medicines and Medical Devices Safety Authority (MEDSAFE) for review by the Standing Committee on Therapeutic Trials (SCOTT) as well as the local Ethics Committee.

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/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.

Source: Arrowhead Pharmaceuticals, Inc.

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