Arrowhead Receives Positive EMA Opinion on Orphan Designation for ARO-AAT for Treatment of Congenital Alpha-1 Antitrypsin Deficiency

June 27, 2018

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 27, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on Arrowhead's application for orphan designation of its RNAi candidate, ARO-AAT, for the treatment of congenital alpha-1 antitrypsin deficiency. ARO-AAT was previously granted orphan drug designation by the United States Food and Drug Administration in February 2018.

Bruce Given, M.D., Arrowhead's chief operating officer and head of R&D, said, "ARO-AAT has the potential to provide patients and physicians with a much-needed treatment option for liver disease associated with alpha-1 antitrypsin deficiency, a rare genetic disorder. The COMP's adoption of a positive opinion for ARO-AAT orphan drug designation is another important milestone for this program, which continues to advance rapidly."

The positive opinion issued by COMP will be sent to the European Commission which is expected to grant the orphan designation within 30 days. Orphan designation in the EU allows Arrowhead to benefit from a number of key incentives, including reduced regulatory fees, protocol assistance, and market exclusivity, to develop a medicine for the treatment of a rare disease affecting not more than five in 10,000 people in the European Union.

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 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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