Arrowhead Pharmaceuticals Doses First Patient in AROAAT2002 Open Label Phase 2 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

December 20, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Dec. 20, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in AROAAT2002, an open-label Phase 2 clinical study of ARO-AAT, the company's second generation investigational RNA interference (RNAi) therapeutic being developed as a treatment for the rare genetic liver disease associated with alpha-1 antitrypsin deficiency (AATD). Arrowhead is also conducting the SEQUOIA Phase 2/3 trial, which is an ongoing potentially pivotal study that began dosing patients in August 2019.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "We are committed to helping the alpha-1 community and would like to thank the participating investigators and patients, who currently have no available treatment options for alpha-1 liver disease other than liver transplant. AROAAT2002 is an important study within our ARO-AAT development program, which provides us with a key opportunity to assess patient response to treatment at various timepoints. This may prove to be helpful for future regulatory and commercial planning as the ARO-AAT development program progresses."

AROAAT2002 (NCT03946449) is a pilot open-label, multi-dose, Phase 2 study to assess changes in a novel histological activity scale in response to ARO-AAT in patients with AATD associated liver disease. Approximately 12 participants will be enrolled in two sequential cohorts. All eligible participants will require a pre-dose biopsy and an end of study biopsy. Treated participants will also be offered the opportunity to continue treatment in an open-label extension (OLE). Including the OLE, changes in the novel histological grading scale will be assessed after 6 months, 12 months, 18 months, and 24 months of treatment with ARO-AAT.

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 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, 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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