Arrowhead Begins Dosing in Phase 1/2 Study of ARO-HBV for Treatment of Chronic Hepatitis B

March 27, 2018

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 27, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first subjects in a Phase 1/2 clinical study of ARO-HBV, which is being developed as a potentially curative therapy for patients with chronic hepatitis B virus (HBV) infection. ARO-HBV is the second clinical candidate developed using Arrowhead's proprietary Targeted RNAi Molecule (TRiM ^{TM}) technology.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead, said, "We are proud of how quickly ARO-HBV moved through preclinical studies and are excited to be starting first-in-human clinical studies. The AROHBV1001 Phase 1/2 clinical study has an innovative design that is intended to assess, in parallel, safety and tolerability in healthy volunteers and multiple-dose anti-viral activity in HBV patients. Our experience with two prior compounds, ARC-520 and ARC-521, provides us with confidence that ARO-HBV, which is designed to target all viral transcripts, including those produced by both cccDNA and HBV DNA integrated into host DNA, has the potential to be a backbone therapy in combinations aimed at achieving a functional cure of chronic HBV infection."

AROHBV1001 (NCT03365947) is a Phase 1/2 study evaluating the safety, tolerability, and pharmacokinetic effects of single-ascending doses (SAD) of ARO-HBV in healthy adult volunteers, and to evaluate the safety, tolerability, and pharmacodynamic effects of multiple-ascending doses (MAD) of ARO-HBV in patients with chronic HBV.

The SAD portion is designed to include up to 5 cohorts of 6 subjects per cohort. Each SAD subject will receive a single-dose administration of either placebo or ARO-HBV at up to 5 dose levels (35, 100, 200, 300, 400 mg). The MAD portion is designed to include up to 8 cohorts of 4 HBV patients per cohort. Each MAD patient will receive 3 doses of ARO-HBV at up to 4 dose levels (100, 200, 300, 400 mg).

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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