



Arrowhead Presents New ARO-HBV Clinical Data Demonstrating HBsAg Reductions at World Gastroenterologists Summit

September 6, 2018

- Data presented from the lowest two dose cohorts (100mg and 200mg ARO-HBV)
- Up to 4.0 log₁₀ reduction in HBsAg observed following three doses of ARO-HBV
- ARO-HBV was generally well-tolerated in HBV patients

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 6, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) will present initial clinical data for ARO-HBV, the company's third generation subcutaneously administered RNA interference (RNAi) therapeutic being developed as a potentially curative therapy for patients with chronic hepatitis B virus (HBV) infection, at the 18th World Gastroenterologists Summit in Auckland, New Zealand. Data will be presented from the eight patients in the lowest two dose cohorts: 100mg and 200mg. The data demonstrate that three monthly doses of ARO-HBV led to a maximum reduction in circulating HBV surface antigen (HBsAg) of 4.0 log₁₀, with mean reductions of approximately 2.0 log₁₀ on day 85 in the 100 mg cohort and 1.4 log₁₀ on day 71 in the 200mg cohort (currently the last complete data point available). All eight patients achieved greater than 1.0 log₁₀ reductions in circulating HBsAg.

Safety data will be presented across all ten patient cohorts (n=40). ARO-HBV was generally well-tolerated with generally mild and self-limiting injection site adverse events being the most common reported event in chronic HBV patients, occurring in around 10% of injections. The other most commonly reported events included symptoms consistent with upper respiratory tract infection and headache.

These results represent the first clinical data presented on ARO-HBV, which leverages Arrowhead's proprietary Targeted RNAi Molecules (TRiM™) platform. The company intends to submit a late-breaking abstract with additional clinical data to the Liver Meeting®, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD), being held in November 2018.

Bruce Given, M.D., Arrowhead's chief operating officer and head of R&D, said, "These initial results from the first two multiple-ascending dose cohorts of the AROHBV1001 clinical study are encouraging and indicate that ARO-HBV is highly active. In addition, the drug appears to be generally well-tolerated, which is consistent with our experience to date with ARO-AAT, our TRiM™ enabled candidate for the treatment of Alpha-1 liver disease. We intend to submit late-breaking abstracts to the AASLD Liver Meeting for both ARO-HBV and ARO-AAT, and, if accepted, we look forward to presenting more complete data-sets, including additional dose levels and longer follow-up."

Key new data to be presented at the 18th World Gastroenterologists Summit from the AROHBV1001 Phase 1/2 clinical study in patients with chronic HBV who received three monthly doses of ARO-HBV include the following:

- Mean reduction of HBsAg was 2.0 log₁₀ (99%) on day 85 in cohort 2b (100 mg) and 1.4 log₁₀ (96%) on day 71 in cohort 3b (200 mg)
 - These may not represent nadir
- Maximum reduction of HBsAg was 4.0 log₁₀ (99.99%)
- Minimum HBsAg reduction in all patients from cohorts 2b and 3b was 1.2 log₁₀ (93%)
- Activity was demonstrated in all patient types (HBeAg pos/neg, NUC naïve/treated)
- ARO-HBV appeared to be generally well-tolerated

The keynote presentation, titled "Hepatitis B in focus: new biology, new targets and real hope for finite therapy," will be delivered by Dr. Given on September 7 at 09:45 a.m. NZST. A copy of the presentation (see slides 21-23) can be accessed on the [Events and Presentations](#) page under the Investors section of the Arrowhead website.

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 evaluating the safety, tolerability, and pharmacodynamic effects of multiple-ascending doses (MAD) of ARO-HBV in patients with chronic HBV. Dosing in the SAD portion of the study is complete, and included five cohorts at dose levels of 35, 100, 200, 300, and 400 mg. Dosing in the MAD portion of the study is ongoing, and includes ten cohorts receiving three doses of ARO-HBV either weekly, bi-weekly, or monthly, and includes dose levels of 100, 200, 300, and 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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