Arrowhead Presents Clinical Data on JNJ-3989 (ARO-HBV) at The International Liver Congress™

April 12, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Apr. 12, 2019-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced the presentation of clinical data from an ongoing Phase 1/2 study (AROHBV1001) of JNJ-3989 (formerly ARO-HBV), a third-generation subcutaneously administered RNA interference (RNAi) therapeutic candidate being developed as a potential treatment for patients with chronic hepatitis B virus (HBV) infection, at The International Liver Congress™ 2019 (ILC), the annual meeting of the European Association for the Study of the Liver (EASL).

Arrowhead entered into a license agreement in October 2018 with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, to develop and commercialize ARO-HBV.

Key results from this interim analysis include the following:

- JNJ-3989 rapidly reduced hepatitis B surface antigen (HBsAg) in patients that had 24 weeks or more of HBsAg assay results (n=40) to thresholds possibly associated with improved chances of HBsAg seroclearance[1] in many patients, after only 3 doses
    - 100% of patients (40 of 40) achieved ≥1.0 Log10 IU/mL HBsAg reduction
    - 88% of patients (35 of 40) achieved HBsAg <100 IU/mL
    - 43% of patients (17 of 40) achieved HBsAg <10 IU/mL
    - 13% of patients (5 of 40) achieved HBsAg <1 IU/mL
- JNJ-3989 reduced all measurable viral products, including HBsAg in hepatitis B e-antigen (HBeAg) positive or HBeAg negative patients
- JNJ-3989 administered subcutaneously was well tolerated at doses up to 400 mg in all chronic hepatitis B (CHB) patients in cohorts 2b-11 (n=56)
    - 168 total doses administered to 56 CHB patients (cohorts 2b through 11)
    - No drug related serious adverse events (SAE) reported
        - Unrelated SAE of menorrhagia
        - Unrelated SAE of anxiety/depression
    - All patients received all 3 scheduled doses; No dropouts
    - No dose related pattern of adverse changes in laboratory values (e.g. ALT, AST, total bilirubin, creatinine)
    - 17 total AEs at injection site (10% of injections) reported (e.g. erythema, tenderness, bruising), all were mild

Oral Presentation Details:

Short term RNA interference (RNAi) therapy in chronic hepatitis B (CHB) using JNJ-3989 brings majority of patients to HBsAg <100 IU/ml

- Presentation Reference: PS-080
- Session: Parallel session: Hepatitis B - drug development
- Session Date and Time: April 12, 2019 at 5:45 p.m. CET
- Authors: Man-Fung Yuen, *et al.*

Additional details, including the presentation abstract, can be found on the ILC website at https://ilc-congress.eu/. A copy of presentation materials can be accessed by visiting the Events section under the Investors tab of the Arrowhead website.

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

Hepatitis B infection is a life-threatening viral infection of the liver, which can cause cirrhosis — scarring of liver tissue — and liver cancer if the infection becomes chronic. The World Health Organization cites that hepatitis B is a global public health problem with 257 million people living with the disease, resulting in 887,000 deaths in 2015.[2] While a preventive vaccine is available, cure rates for those infected remain low and most patients will endure lifelong therapy.

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.

Source: Arrowhead Pharmaceuticals, Inc.

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[1] Jeng et al. 2018 Hepatology 68:425-434

[2] World Health Organization (WHO). Hepatitis B. July 2017. Available at: http://www.who.int/mediacentre/factsheets/fs204/en/

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Source: Arrowhead Pharmaceuticals, Inc.

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