Arrowhead and Collaborator Janssen Present Phase 2 Clinical Data for Investigational Hepatitis B Regimens at The Liver Meeting® 2019

November 8, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 8, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced Phase 2 clinical data on a double combination of JNJ-3989 (formerly ARO-HBV) and a nucleos(t)ide analog (NA), and the first clinical data on a triple combination of JNJ-3989, JNJ-6379, and an NA, with collaborator Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson. The data is being presented in two poster presentations at The Liver Meeting®, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD).

Janssen is currently conducting Phase 2b studies of JNJ-3989 in combination with JNJ-6379 and an NA for the treatment of chronic hepatitis B virus infection. Arrowhead entered into a license and collaboration agreement with Janssen in October 2018 to develop and commercialize JNJ-3989.

JNJ-3989 is a liver-targeted investigational antiviral therapeutic for subcutaneous injection designed to treat chronic HBV (CHB) infection via the ribonucleic acid interference (RNAi) mechanism. JNJ-6379 is an investigational orally administered capsid assembly modulator of the class that forms normal capsid structures (CAM-N).

Poster Details:

Dose Response with the RNA Interference Therapy JNJ-3989 Combined with Nucleos(t)ide Analogue Treatment in Expanded Cohorts of Patients with Chronic Hepatitis B

- Publication Number: 0696
- Session: Hepatitis B - Therapeutics: New Agents
- Session Date and Time: November 8, 2019 from 8:00 AM to 5:30 PM EST
- Location: Hynes Convention Center, Hall B
- Authors: Dr. Edward J. Gane, *et al.*

Key points presented include the following:

- In the AROHBV1001 study in CHB patients, JNJ-3989 in combination with an NA had strong activity against hepatitis surface antigen (HBsAg), HBV DNA and HBV RNA. Reductions in hepatitis B e antigen (HBeAg) and hepatitis B core-related antigen (HBcrAg) were generally less pronounced
    - HBsAg reductions were similar in HBeAg positive and HBeAg negative patients
    - Expanded cohorts with 100–400 mg JNJ-3989 confirmed previous findings that HBsAg declines were similar with these doses; 97% (31/32) of these patients achieved a ≥1.0 log10 (90%) reduction in HBsAg
    - The 25 mg and 50 mg JNJ-3989 doses were active in reducing HBsAg, and appeared less effective than higher doses
    - HBsAg responses with JNJ-3989 are consistent with its ability to silence HBV RNA from cccDNA and host-integrated viral DNA (which is a major source of HBsAg in certain CHB populations)
    - JNJ-3989 was well tolerated at doses up to 400 mg Q4w for three doses.
- Overall, JNJ-3989 demonstrated anti-HBV characteristics desirable for an effective RNAi therapy

First Clinical Experience with RNA Interference [RNAi]-Based Triple Combination Therapy in Chronic Hepatitis B (CHB): JNJ-73763989 (JNJ-3989), JNJ-56136379 (JNJ-6379) And a Nucleos(T)ide Analogue (NA)

- Publication Number: LP4
- Session: Late-Breaking Poster Session
- Session Date and Time: November 11, 2019 from 8:00 AM to 5:30 PM EST
- Location: Hynes Convention Center, Hall B
- Authors: Dr. Man-Fung Yuen, *et al.*

Key points presented include the following:

- This is the first study to investigate the safety and efficacy of a triple combination of an RNAi (JNJ-3989 200 mg 3x Q4w, three doses), a CAM-N (JNJ-6379 250 mg daily for 12 weeks) and an NA (daily) in patients with CHB
- This triple combination was well tolerated, and all CHB patients achieved robust reductions in HBsAg, HBV DNA, and HBV RNA. Reductions in HBeAg and HBcrAg were generally less pronounced during the dosing period
    - All patients (n=12) achieved a ≥1.0 log10 IU/mL (90%) reduction (nadir ranged from –1.01 to –2.26 log10 IU/mL) in HBsAg
    - HBsAg reductions were similar in HBeAg positive and HBeAg negative patients
- Studies of longer duration with this triple combination are underway aimed at assessing functional cure rates in patients with CHB

Copies of the poster presentations can be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

Hepatitis B infection is a life-threatening viral infection of the liver, which can cause cirrhosis — scarring of liver tissue — and liver cancer after prolonged period of chronic infection. The World Health Organization cites that hepatitis B is a global public health problem affecting 292 million people worldwide.[1] 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] The Polaris Observatory Conservatory. Global prevalence, treatment, and prevention of hepatitis B. The Lancet Gastroenterology & Hepatology. VOLUME 3, ISSUE 6, P383-403, JUNE 01, 2018

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Source: Arrowhead Pharmaceuticals Inc.

Arrowhead Pharmaceuticals, Inc.
Vince Anzalone, CFA
626-304-3400
ir@arrowheadpharma.com

Investors and Media:
LifeSci Advisors, LLC
Brian Ritchie
212-915-2578
britchie@lifesciadvisors.com
www.lifesciadvisors.com