Arrowhead Announces First Patient Enrolled in Phase 1 Trial Evaluating HZN-457 for the Treatment of Gout

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- Arrowhead Earns $15 million Milestone from Horizon Therapeutics plc

PASADENA, Calif.--(BUSINESS WIRE)--Dec. 8, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that Horizon Therapeutics plc (Nasdaq: HZNP) has enrolled the first subject, earning Arrowhead a $15 million milestone payment, in a Phase 1 randomized, placebo-controlled trial to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of its development-stage medicine HZN-457, previously known as ARO-XDH. HZN-457 is a small interfering RNA (siRNA) candidate designed to treat gout by silencing liver xanthine dehydrogenase. The development of HZN-457 is part of a global collaboration and license agreement that the Company entered in 2021 with Horizon.

“Our partnership with Horizon has moved forward rapidly and we are excited that HZN-457, formerly ARO-XDH, has begun clinical studies,” said Christopher Anzalone, Ph.D., Arrowhead’s president and CEO. “Horizon is a clear leader in gout and we look forward to continuing to work with them to help bring this potentially important new medicine to the patients who need it.”

“As a clinical and commercial leader in gout, Horizon understands the critical need to develop innovative approaches and advance novel treatments to reach more patients who live with this disease,” said Elizabeth H.Z. Thompson, Ph.D., executive vice president, research and development, Horizon. “The HZN-457 Phase 1 clinical trial marks an important milestone for Horizon to advance our research and development in this space, fostered by our partnership with Arrowhead.”

Gout is a serious and painful form of inflammatory arthritis that is caused by excess uric acid. In the United States, there are more than 9 million gout patients and approximately a third are treated with oral urate-lowering therapies.[1,2] However, a meaningful portion of those patients do not respond sufficiently and continue to experience painful and debilitating gout symptoms.[3] High uric acid levels, if left untreated or undertreated, can lead to acute and persistent inflammatory arthritis,[4] bone and joint damage, exacerbate comorbidities, and severely impact patients’ lives. [5] Preclinical studies suggest that HZN-457 has the potential to maintain lower uric acid levels and offer several benefits above standard of care for the treatment of gout.

Approximately 56 healthy volunteers are expected to be enrolled sequentially into ascending dose cohorts and administered HZN-457 subcutaneously as a single dose. The primary endpoint is safety and tolerability. Secondary endpoints include the assessment of plasma and urine pharmacokinetic parameters and changes from baseline in uric acid levels.

About HZN-457

HZN-457 is a small interfering RNA (siRNA) medicine candidate conjugated to N-acetylgalactosamine (GalNAc) that selectively targets and silences xanthine dehydrogenase expression in the liver, which produces uric acid. The development of HZN-457 is through an exclusive collaboration between Arrowhead and Horizon Therapeutics plc.

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. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as “may,” “will,” “expect,” “believe,” “anticipate,” “hope,” “intend,” “plan,” “project,” “could,” “estimate,” or “continue” are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects, or benefits of our collaborations with other companies, or other characterizations of future events or circumstances are forward-looking statements. 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 numerous factors and uncertainties, including the impact of the ongoing COVID-19 pandemic on our business, the safety and efficacy of our product candidates, decisions of regulatory authorities and the timing thereof, 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, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. 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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