Arrowhead Pharmaceuticals and Horizon Therapeutics Announce Collaboration to Develop RNAi Therapeutic for Uncontrolled Gout

June 21, 2021

- Arrowhead to receive $40 million upfront payment

- Horizon to receive a worldwide exclusive license for an investigational short interfering RNA (siRNA) therapeutic targeting xanthine dehydrogenase (XDH)

PASADENA, Calif. & DUBLIN--(BUSINESS WIRE)--Jun. 21, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) and Horizon Therapeutics plc (NASDAQ: HZNP) today announced a global collaboration and license agreement for ARO-XDH, a previously undisclosed discovery-stage investigational RNA interference (RNAi) therapeutic being developed by Arrowhead as a potential treatment for people with uncontrolled gout.

Gout is a serious and painful form of arthritis that is caused by excess uric acid in the blood. In the United States, there are more than nine million gout patients and approximately a third of those patients are treated with oral urate-lowering therapies. However, a meaningful portion of treated patients do not respond sufficiently to treatment and therefore continue to experience painful and debilitating gout symptoms. XDH represents a clinically validated target that is the primary source of serum uric acid (sUA). High levels of sUA, if left untreated or undertreated, can potentially lead to serious long-term or even permanent damage to the bones, joints and organs.

Under the terms of the agreement, Arrowhead will conduct all activities through preclinical stages of development of the siRNA therapeutic. Horizon will receive a worldwide exclusive license to the therapeutic and will be wholly responsible for clinical development and commercialization. Arrowhead will receive $40 million as an upfront payment from Horizon and is eligible to receive up to $660 million in potential development, regulatory and commercial milestones, and is further eligible to receive royalties in the low- to mid-teens range on net product sales.

“We believe Horizon’s expertise in the clinical development and commercialization of therapies for gout makes them an ideal partner. We look forward to working closely with Horizon as we advance this potential new therapy for patients in need,” said Christopher Anzalone, Ph.D., Arrowhead’s president and CEO. “This collaboration with Horizon also represents further expansion of our rapidly growing pipeline of investigational RNAi therapeutics that utilize Arrowhead’s proprietary Targeted RNAi Molecule (TRiM ^{TM}) platform.”

“As a commercial and clinical leader in gout, we understand the disease and its impact, and that there is still a tremendous unmet treatment need,” said Tim Walbert, chairman, president and CEO, Horizon. “The collaboration with Arrowhead adds an additional program to our research and development focus to discover and develop new, innovative medicines that continue to improve gout treatment across the continuum of care.”

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.

About Horizon

Horizon is focused on the discovery, development and commercialization of medicines that address critical needs for people impacted by rare, autoimmune and severe inflammatory diseases. Our pipeline is purposeful: we apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives. For more information on how we go to incredible lengths to impact lives, please visit www.horizontherapeutics.com and follow us on Twitter, LinkedIn, Instagram and Facebook.

Arrowhead 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,” “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, expectations for our product pipeline, prospects or benefits of our collaborations, or potential 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, 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.

Horizon Forward Looking Statements:

This press release contains forward-looking statements, including, but not limited to, statements related to expected activities and payments under the collaboration between Horizon and Arrowhead, and the potential benefits of the therapeutic candidate as a treatment for uncontrolled gout. These forward-looking statements are based on Horizon’s current expectations and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which

include, without limitation, risks and uncertainties associated with development, regulatory approval and commercialization of novel therapeutic candidates, the fact that the collaboration is subject to early termination and risks associated with the COVID-19 global pandemic. For a further description of these and other risks facing Horizon, please see the risk factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon undertakes no obligation to update or revise these statements, except as may be required by law.

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