Arrowhead Announces ARO-DUX4 as First Muscle Targeted RNAi Candidate Using TRiMTM Platform

April 15, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Apr. 15, 2021-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced ARO-DUX4 as Arrowhead's first muscle targeted investigational RNAi therapeutic candidate to utilize its proprietary Targeted RNAi Molecule (TRIM ^{TM}) platform. ARO-DUX4 is designed to target the gene that encodes human double homeobox 4 (DUX4) protein as a potential treatment for patients with facioscapulohumeral muscular dystrophy (FSHD). Pending abstract acceptance, Arrowhead intends to present preclinical data on ARO-DUX4 at the 28th Annual FSHD Society International Research Congress being held virtually on June 24-25, 2021. Arrowhead is currently conducting IND/CTA enabling toxicology studies and intends to file for regulatory clearance in the third quarter of 2021 to begin clinical studies of ARO-DUX4.

Chris Anzalone, Ph.D., president and chief executive officer at Arrowhead, said: "ARO-DUX4 represents further expansion of our pipeline into diseases of the skeletal muscle, which is the fourth cell type that we may potentially be able to target with our proprietary TRiM^{TM} platform. In addition, DUX4 fits perfectly with our strategy not only to bring RNAi outside the liver, but also to select gene targets that we believe are clear causes of specific diseases and for which there is strong biologic and genetic validation. We believe our preclinical data, which we intend to present at the FSHD Society International Research Congress in June pending abstract acceptance, highly support progressing ARO-DUX4 into clinical studies. In our various animal models, ARO-DUX4 reduced DUX4 expression by greater than 70%, prevented body weight loss associated with tamoxifen-induced DUX4 expression, and prevented loss of muscle function."

FSHD is an autosomal dominant disease associated with the failure to maintain complete epigenetic suppression of DUX4 expression in differentiated skeletal muscle, leading to overexpression of DUX4, which is myotoxic and can lead to muscle degeneration. As DUX4 expression is recognized as the cause of muscle pathology in FSHD patients, Arrowhead believes that the selective targeting and knockdown of DUX4 using RNAi may prevent or reverse downstream myotoxicity and lead to muscle repair and improvement in muscle function in patients. There are currently no effective treatments specifically for FSHD.

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," "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 businesses, 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 continuing impact of the COVID-19 pandemic, 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.

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