Arrowhead Pharmaceuticals Files for Regulatory Clearance to Initiate a Phase 1/2 Study of ARO-DUX4 for Facioscapulohumeral Muscular Dystrophy

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PASADENA, Calif.--(BUSINESS WIRE)--Jul. 17, 2023-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it has filed an application for clearance to initiate a Phase 1/2 clinical trial of ARO-DUX4, the company's investigational RNA interference (RNAi) therapeutic being developed as a potential treatment for patients with facioscapulohumeral muscular dystrophy (FSHD). ARO-DUX4 is the first clinical candidate utilizing Arrowhead's proprietary Targeted RNAi Molecule (TRiM ^{TM}) platform to target disease associated genes in skeletal muscle.

"FSHD is a debilitating disease caused by the aberrant expression of the DUX4 gene in skeletal muscle. ARO-DUX4 engages the RNA interference pathway to selectively inhibit DUX4 expression and is a promising strategy to reverse the downstream myotoxicity of DUX4 and potentially enable stabilization or improvement in muscle function in patients. We believe ARO-DUX4 has the potential to be an impactful new medicine for patients," said Chris Anzalone, Ph.D., president and CEO of Arrowhead. "As previously communicated, we are in discussions with external parties regarding the most appropriate path for the clinical development and commercialization of ARO-DUX4 with the goal of bringing this promising product candidate to patients in need as expeditiously as possible."

An application for approval of the clinical trial was submitted to the New Zealand Medicines and Medical Devices Safety Authority for review by the Standing Committee on Therapeutic Trials. Pending clearance, Arrowhead intends to proceed with ARODUX4-1001, a Phase 1/2a dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-DUX4 in adult patients with FSHD type 1. The study is designed to enroll up to 52 subjects.

About Facioscapulohumeral Muscular Dystrophy

Facioscapulohumeral muscular dystrophy (FSHD) is a debilitating, muscle-wasting disease characterized by progressive asymmetrical weakness and fatty infiltration of skeletal muscles in the face, shoulders, upper arms, abdomen, or lower leg and is the third most common form of muscular dystrophy. There is currently no approved therapy for FSHD.

About ARO-DUX4

ARO-DUX4 is an investigational RNAi therapeutic designed to reduce expression of the gene that encodes the human double homeobox 4 (DUX4) protein as a potential treatment for FSHD. 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. Expression of downstream DUX4 associated genes over time leads to muscle fatty infiltration and progressive muscle weakness. As DUX4 overexpression is the cause of muscle pathology in FSHD patients, it is expected that the selective knockdown of DUX4 messenger RNA transcripts using RNA interference will inhibit DUX4 translation, thereby preventing or reversing downstream myotoxicity and allowing muscle repair and partial or complete return of normal muscle function in patients.

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," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology 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 forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. 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.

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