



Arrowhead Pharmaceuticals Initiates Phase 1/2a Study of ARO-DM1 for Treatment of Type 1 Myotonic Dystrophy

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- Preclinical data show ARO-DM1 reduces muscular DMPK expression and corrects spliceopathies, which could lead to improved muscle strength and function

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 8, 2024-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) announced today that it has dosed the first subjects in a Phase 1/2a double-blinded, placebo-controlled, dose-escalating study ([NCT06138743](#)) to evaluate single and multiple ascending doses of ARO-DM1, the company's investigational RNA interference (RNAi) therapeutic, in up to 48 subjects with type 1 myotonic dystrophy (DM1). DM1 is the most common adult-onset muscular dystrophy.

Patients with DM1 have muscle weakness and wasting, myotonia, cataracts, and often develop cardiac conduction abnormalities. Additionally, patients may become physically disabled and have a shortened life span. There is currently no approved disease-modifying therapy for DM1. Treatments have focused on symptomatic management, including physical therapy, exercise, ankle-foot orthoses, and assistive devices, such as wheelchairs.

ARO-DM1 is designed to reduce expression of the dystrophin myotonia protein kinase (DMPK) gene in the muscle. Pathogenesis of DM1 is driven by an expanded CUG trinucleotide repeat in the 3'-untranslated region of DMPK transcripts. These abnormal transcripts cause mis-regulated splicing, known as spliceopathy, for certain messenger RNAs which are directly linked to the clinical manifestations of DM1.

Preclinical data recently presented at the 2024 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference showed that in nonhuman primates, ARO-DM1 achieved greater than 80% silencing of DMPK in skeletal muscle, which was maintained for longer than 85 days. In a mouse model of DM1 harboring a pathogenic DMPK transgene, a modified species-specific version of ARO-DM1 (S-ARO-DM1) decreased the DMPK-CUG expression and corrected the spliceopathies.

Presentation materials may be accessed on the [Events and Presentations](#) page under the Investors section of the Arrowhead website.

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 X (formerly Twitter) at [@ArrowheadPharma](#) or on [LinkedIn](#). 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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