ARTHEx Biotech Announces First Patient Dosed in Phase I-IIa ArthemiR™ Trial for Myotonic Dystrophy Type 1 (DM1)

-ATX-01 is the industry's first anti-microRNA therapeutic to be investigated in DM1 –

Valencia, Spain, October 18, 2024 – ARTHEx Biotech S.L., a clinical-stage biotechnology company focused on developing innovative medicines through the modulation of gene expression, announced today that the first participant has been dosed in the Randomized Placebo Controlled Double Blind Phase I-IIa ArthemiR™ Trial of ATX-01 for Myotonic Dystrophy Type 1 (DM1). DM1 is a devastating rare neuromuscular disorder that causes muscle weakness and other life-limiting complications. There are currently no disease-modifying treatments approved for DM1.

ATX-01, the industry's first anti-microRNA therapeutic to be evaluated for DM1, targets microRNA 23b (miR-23b) which is known to be a regulator of the expression of muscle blind like (MBNL) proteins that are essential for the correct expression (splicing) of many messenger ribonucleic acids (mRNAs) and hence proteins, in the body. DM1 pathogenesis is caused by sequestration of MBNL by toxic dystrophia myotonica protein kinase (DMPK) mRNA in the nucleus, and by translational repression of MBNL production caused by miR-23b overexpression, both of which lead to a net decrease of MBNL available to exert its usual regulatory functions. ATX-01 is the only therapeutic agent that acts with a dual mechanism of action, by (1) increasing MBNL production, and (2) by destabilizing the toxic DMPK foci leading to a reduction of DMPK mRNA and release of sequestered MBNL. Both factors contribute to a general increase of active MBNL levels and splicing correction.

"Since the founding of ARTHEx, we have been working tirelessly to achieve our goal of creating a new therapeutic option for persons living with DM1 and their families. We are proud to have reached a major milestone in our company's journey with the dosing of the first participant in our ArthemiR™ trial," said Dr. Frédéric Legros, Chairman and CEO of ARTHEx. "We believe that the market opportunity for a new DM1 therapy is very attractive, given the important unmet medical need and the lack of an approved agent that is disease-modifying."

"ATX-01 is a very unique molecule – it was discovered and developed entirely in-house by ARTHEx scientists through years of engineering and execution, and we are excited about the potential of its dual mechanism of action to improve the lives of patients with DM1," said Dr. Beatriz Llamusí, Chief Scientific Officer and Co-Founder of ARTHEx.

The ArthemiR™ trial is a global Phase I-IIa double-blind, placebo-controlled, single and multiple ascending dose study in participants with classic DM1. The primary objective is to determine the safety and tolerability of ATX-01 in DM1 participants. ARTHEx will also investigate target engagement at the muscle level through biomarkers, including MBNL levels and RNA splicing index. The clinical endpoints from the trial will include measures related to muscle function, patient-reported outcomes, and quality of life measures. The plan is to enroll fifty-six participants to allow an assessment of ATX-01's performance in this population.

Dr. Judy Walker, Chief Medical Officer of ARTHEx, added, “The ArthemiR™ trial is designed to assess the safety and tolerability of ATX-01 in persons with DM1, as well as its activity on the disease pathophysiology and clinical outcomes. We are very pleased with the non-clinical safety profile of ATX-01 and are excited about moving this agent to clinic as it offers a fresh approach to the disease due to its dual mechanism of action. We sincerely hope that ATX-01 will lead to functional benefits for patients with DM1, offer a well tolerated safety profile, and improve quality of life for those with the condition. We look forward to continuing to enroll participants and initiating more sites across the globe.”

Dr. Valeria Sansone, Professor of Neurology at the University of Milan, commented, "Dosing the first participant in the ArthemiR trial is a significant milestone that represents years of research and development for DM1. Initiating new clinical studies with novel treatment options like ATX-01 may be critical for patients, families and the broader community to gain additional knowledge which may help lead to better clinical outcomes and possibly an approved treatment. I am excited to evaluate ATX-01's dual mechanism of action in the ArthemiR trial."

About ATX-01

ATX-01 is an antimiR oligonucleotide designed to target microRNA 23b (miR-23b), which is involved in the pathogenesis of DM1. It has been demonstrated, in human DM1 myoblast cell lines and in two murine models, that ATX-01 has a unique, dual mechanism of action which reduces toxic DMPK mRNA and increases MBNL protein production.

ATX-01 was discovered through ARTHEx’s in-house discovery engine, which was built to identify, design and optimize novel gene expression modulators and ensure their preferential delivery to target tissues affected by the disease.

About Myotonic Dystrophy Type 1 (DM1)

Myotonic dystrophy type 1 (DM1) is a highly disabling disease affecting more than one million people worldwide. The condition affects muscles and other tissues (causing respiratory problems, fatigue, hypersomnia, cardiac abnormalities, severe gastrointestinal complications, and cognitive and behavioral impairment). Most commonly, it manifests during adulthood (classic DM1). Although signs and symptoms vary among affected individuals, with progression of the disease, DM1 patients experience a reduction in the ability to perform activities of daily living. Moreover, patients have a significantly shortened lifespan and there is currently no approved treatment to slow the progression of the disease.

About ARTHEx Biotech

ARTHEx Biotech is a clinical-stage biotechnology company focused on developing innovative medicines through the modulation of gene expression. The Company’s lead investigational compound, ATX-01, is being evaluated for the treatment of myotonic dystrophy type 1 (DM1), a rare neuromuscular disorder, in the Phase I-IIa ArthemiR™ trial. ArthemiR trial is co-funded by EIC Accelerator program under the Grant Agreement Nº 190181217. ARTHEx is advancing its in-house discovery engine to identify and develop nucleic acid-based therapies for other disorders with high unmet medical need, including genetically-driven diseases. The Company headquarters are in Valencia, Spain.

For more information, please visit www.arthexbiotech.com and engage with us on LinkedIn.

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