



## **ARTHEX Biotech Upsizes Series B Financing Round to \$87M to Advance Lead Program ATX-01 in Myotonic Dystrophy Type 1 and Expand Pipeline of Targeted RNA Medicines**

*- Extension financing led by new investor Bpifrance, with renewed participation from all existing shareholders, AdBio Partners, CDTI Innovación, Columbus Venture Partners, European Innovation Council (EIC), Hadean Ventures, Invivo Partners and Sound Bioventures -*

*- Proceeds to support further clinical development of lead program ATX-01 in Myotonic Dystrophy Type 1, a unique therapy currently in Phase I-IIa, targeting miR23b -*

*- Funding to also advance a broader pipeline of targeted RNA medicines across areas of high unmet need in muscular, CNS, cardiac conditions -*

Valencia, Spain, September 17<sup>th</sup>, 2025 – ARTHEX Biotech S.L., a clinical-stage biotechnology company focused on developing targeted RNA medicines for rare genetic neuromuscular disorders, today announced the successful closing of its upsized Series B financing with new investor Bpifrance, bringing the total size of the funding round to \$87 million.

The Series B extension financing was led by Bpifrance, as part of both Large Venture and InnoBio investment strategies, and joined by all existing shareholders, including AdBio Partners, CDTI Innovación (through its Invierte program), Columbus Venture Partners, European Innovation Council (EIC), Hadean Ventures, Invivo Partners and Sound Bioventures.

Proceeds from the financing will be used to further advance the global clinical development of ARTHEX's lead program, ATX-01 for Myotonic Dystrophy Type 1 (DM1), including the ongoing interventional Phase I/IIa Arthemir<sup>TM</sup> study and the preparation for an open-label extension to support a registrational study.

ATX-01 is an anti-miR oligonucleotide designed to inhibit microRNA23b (miR-23b), which is a natural repressor of MBNL protein expression. In DM1 patients, loss of MBNL protein function is caused by (1), reduced expression of MBNL proteins due to miR-23b upregulation and (2) MBNL sequestration in toxic DMPK mRNA, which leads to a spliceopathy, and is the cause of symptomatology in DM1 patients.

By inhibiting miR-23b, ARTHEX has demonstrated that ATX-01 increases MBNL production and decreases foci formation and toxic DMPK mRNA. This highly differentiated dual mechanism of action leads to a significant increase of free MBNL, improving splicing abnormalities, and ultimately restoring function in animal models.

Laurent Higuieret, Deputy Director at Bpifrance's Large Venture Fund, stated: "We believe ATX-01 could be a game-changer for patients suffering from DM1 on the basis of novel science and impressive data generated so far. We are excited to partner with ARTHEX as the company reaches clinical proof-of-concept stage and look forward to supporting Frederic and team in their efforts to build a leading franchise of precision RNA medicines."

Benoit Barteau, Investment Director at Bpifrance's InnoBio funds, added: "The approach developed by ARTHEx for DM1 aiming at targeting miR-23b has demonstrated compelling in vitro and in vivo results. The dual mechanism of action of ATX-01 offers real potential to be the best-in-class treatment for DM1. In addition, the Company's delivery platform enables uptake into multiple tissues affected by DM1, allowing ATX-01 to go beyond the muscle, treating the whole disease and not just the symptomatology. We are eager to see initial clinical data in 2026."

Frédéric Legros, Chairman and CEO, commented, "This financing marks an important milestone for ARTHEx and underscores the strength of our approach in DM1 and our emerging delivery platform, with its potential to deliver nucleic acid-based therapies beyond muscle. We are well positioned to advance ATX-01 toward a registrational study for DM1, while continuing to expand our pipeline across areas of high unmet need in muscular, CNS, cardiac conditions."

"Arthex has made significant progress over the past years, advancing both its science and its clinical program. All investors are very pleased to continue supporting the company, and we believe that reinforcing its position through this financing is an important step to enable the next phase of its development". said Jose Mesa, Partner at Columbus Venture Partners, initial lead investor of the series B.

### **About ATX-01 and the ArthemiR™ Study**

ATX-01 is an oleic acid-conjugated antimiR oligonucleotide with preferential delivery to target tissues (muscle & brain) designed to inhibit microRNA 23b (miR-23b), which is a natural repressor of MBNL protein expression. In DM1 patients, loss of MBNL protein function caused by (1), reduced expression of MBNL protein due to miR-23b upregulation and (2) MBNL sequestration in toxic DMPK mRNA, lead to a spliceopathy, which is the cause of symptomatology in DM1 patients.

In human DM1 myoblast cell lines obtained from patients with a wide range of CTG repeat lengths, ATX-01 increased MBNL protein expression and significantly reduced toxic DMPK mRNA, correcting critical molecular defects such as spliceopathy.

Beneficial effects were also seen in both the HSA<sup>LR</sup> and DMSXL mouse models, demonstrating molecular and functional improvements.

The ArthemiR™ study is a randomized, placebo-controlled, double-blind single (SAD) and multiple ascending dose (MAD) study evaluating ATX-01 in adults with DM1.

ATX-01 has received Orphan Drug Designation for ATX-01 in DM1 from the US FDA and European authorities, as well as Rare Pediatric Disease (RPD) Designation from the FDA.

### **About ARTHEx Biotech**

ARTHEx Biotech is a clinical-stage company developing targeted RNA medicines designed to precisely modulate gene expression. Its proprietary Stratos™ platform pairs selective oligonucleotides with tissue-specific delivery to reach skeletal muscle, heart, and brain. Its lead program, ATX-01, is in clinical evaluation for myotonic dystrophy type 1 (DM1), a rare neuromuscular disorder, in the Phase I/IIa

ArthemIR™ trial. Building on this foundation, ARTHEx is advancing a pipeline of therapies for additional areas of high unmet need across muscular, CNS, cardiac, and pulmonary diseases.

The Company headquarters are in Valencia, Spain.

For more information on ArthemIR™, please visit <https://www.arthemir.com> or <https://clinicaltrials.gov>. For more information, please visit [www.arthexbiotech.com](http://www.arthexbiotech.com) and engage with us on LinkedIn.

### **About Bpifrance and its InnoBio and Large Venture funds**

Bpifrance is the French national investment bank: it finances businesses - at every stage of their development - through loans, guarantees, equity investments and export insurances. Bpifrance also provides extra financial services (training, consultancy) to help entrepreneurs meet their challenges (innovation, export).

InnoBio funds are investment funds dedicated to life sciences, managed by Bpifrance, which is also one of the LPs alongside pharmaceutical companies and institutional investors. These funds aim to invest in companies developing innovative products, close to or in early clinical development, with the objective of bringing them to clinical proof of concept. InnoBio funds take minority equity stake in companies and can lead or co-lead the investment rounds.

Large Venture - the late-stage VC arm of Bpifrance - is a €2.5 billion fund dedicated to fast-growing, highly innovative startups looking to accelerate organic or external growth. Large Venture was incepted in 2013 with the mission to bring the most promising French technologies from the lab to the market, and ultimately to profitability. Large Venture invests in private and public companies across three main sectors: healthtech and life sciences, digital and greentech.

For more information, please visit: [www.bpifrance.com](http://www.bpifrance.com) – Follow us on X : @Bpifrance - @BpifrancePresseand LinkedIn

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