



uniQure Announces Favorable Recommendation from Independent Data Monitoring Committee for its Phase I/II EPISOD1 Clinical Trial of AMT-162 for the Treatment of SOD1-ALS

January 30, 2025

~ Independent Data Monitoring Committee Recommends Proceeding with Dose Escalation After Planned Safety Assessment of First Dose Cohort ~

~ Company Expects to Initiate Enrollment of Second Dose Cohort in the First Quarter of 2025 ~

LEXINGTON, Mass. and AMSTERDAM, Jan. 30, 2025 (GLOBE NEWSWIRE) -- [uniQure](#) N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that the Independent Data Monitoring Committee (IDMC) for EPISOD1, uniQure's Phase I/II clinical trial of AMT-162, an investigational gene therapy for amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1) gene, has met and reviewed 28-day safety data from the first study cohort. The IDMC's review identified no significant safety concerns and recommended proceeding with enrollment in the second cohort.

"We are pleased with the positive outcome of this initial IDMC meeting, which marks a meaningful step in the clinical development of AMT-162 for SOD1-ALS," stated [Walid Abi-Saab, M.D., chief medical officer of uniQure](#). "We will continue to advance the study and look forward to proceeding with dose-escalation in the second cohort of patients."

AMT-162 is an investigational AAVrh10-based gene therapy that expresses a miRNA designed to knock down the expression of the mutated SOD1 protein. Patients with SOD1-ALS express a misfolded SOD1 protein. This misfolded protein is toxic to motor neurons causing degeneration that, over time, leads to muscle weakness, loss of function and eventually death. AMT-162 may offer a novel one-time, intrathecally administered approach to slowing or halting the progression of SOD1-ALS.

About the Phase I/II Clinical Program of AMT-162

EPISOD1 is a Phase I/II multi-center, open-label trial of AMT-162 for the treatment of SOD1-ALS being conducted in the United States consisting of three dose-escalating cohorts with up to four patients each receiving a short course of immunosuppression prior to and after an intrathecal infusion of AMT-162. The trial will explore the safety and tolerability of AMT-162 and will assess exploratory signs of efficacy by measuring neurofilament light chain, a biomarker of neuronal damage, and SOD1 protein. Additional details are available on www.clinicaltrials.gov (NCT06100276).

AMT-162 has been granted both Orphan Drug status and Fast Track designation by the U.S. Food and Drug Administration.

About Amyotrophic Lateral Sclerosis (ALS) caused by mutations in Superoxide Dismutase 1 (SOD1)

SOD1-ALS is a rare, progressive and fatal neurodegenerative disease that results in the loss of motor neurons in the brain and spinal cord. As ALS progresses, individuals experience muscle weakness and atrophy causing them to lose the ability to move their arms and legs, speak, swallow and eventually breathe, causing respiratory failure. Based on a 2021 study in Neuroepidemiology, there are an estimated ~170,000 individuals with ALS globally with SOD1 mutations representing 2% within that population. According to the National Institute of Neurological Disorders and Stroke, the average life expectancy for those diagnosed with ALS is three to five years from onset of symptoms.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The approvals of uniQure's gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represent a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. uniQure is now advancing a [pipeline](#) of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, ALS, Fabry disease, and other severe diseases. www.uniQure.com

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "establish," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "seek," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. Examples of these forward-looking statements include, but are not limited to, statements regarding plans to initiate enrollment in the second dose cohort of the AMT-162 study in the first quarter of 2025 and to proceed with dose escalation along with the potential efficacy profile of AMT-162 through one-time administration with the ability to slow or halt the progression of SOD1-ALS. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, without limitation, risks associated with the clinical results and the development and timing of the Company's programs; the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to approval; the Company's ability to continue to build and maintain the company infrastructure and personnel needed to achieve its goals; the Company's effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of gene therapies; the Company's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; the Company's ability to obtain, maintain and protect its intellectual property; and the Company's ability to fund its operations and to raise additional capital as needed. These risks and uncertainties are more fully described under the heading "Risk Factors" in the Company's periodic filings with the U.S. Securities & Exchange Commission ("SEC"), including its Annual Report on Form 10-K filed February 28, 2024 and its Quarterly Report on Form 10-Q filed November 5, 2024, and in other filings that the Company makes with the SEC from time to time. Given these risks, uncertainties and other

factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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The logo for uniQure, featuring the word "uniQure" in a bold, orange, sans-serif font. The "Q" is stylized with a dot.