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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

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

uniQure, a leading gene therapy company advancing transformative therapies for patients with severe medical needs, 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).

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