uniQure Announces Preliminary Data on the First Cohort in the Phase I/IIa Clinical Trial of AMT-260 in Refractory Mesial Temporal Lobe Epilepsy

June 19, 2026

~ AMT-260 was generally well-tolerated with no serious adverse events observed to date, with early biological signals of potential therapeutic activity ~

LEXINGTON, Mass. and AMSTERDAM, June 19, 2026 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced initial six-month follow-up data on the first, low dose cohort of six patients in its ongoing Phase I/IIa trial of an investigational gene therapy candidate, AMT-260, for the treatment of refractory mesial temporal lobe epilepsy (MTLE). The data will be presented today, Friday, June 19, 2026, at the Epilepsy Foundation Pipeline Conference in Leesburg, VA.

As of the May 29, 2026 data cutoff date, three of six patients in the first, low-dose cohort ($1x10^{12}$ gc/mL) achieved meaningful reductions in disabling seizures during months four through six of follow-up, ranging from a 79% to 100% decline from baseline. The remaining three patients in the low-dose cohort experienced variable changes in disabling seizures during months four through six of follow-up, ranging from a 33% decrease to a 36% increase compared to baseline.

As of the date of the presentation, there have been no Serious Adverse Events (SAEs) related to AMT-260 or the surgical procedure reported. All reported adverse events in the low dose cohort were classified as mild or moderate in severity, with the most common adverse event being headache (N=2). No immunosuppression was required.

"While patient responses have varied, we believe the data generated to date provide preliminary evidence of biological activity of AMT-260," said Walid Abi-Saab, M.D., chief medical officer of uniQure. "Although these findings are based on a limited number of patients and require longer follow-up, the favorable tolerability profile and observed responses support continued evaluation of AMT-260."

Enrollment is ongoing in a second, higher dose cohort ($3x10^{12}$ gc/mL), expected to consist of six patients with enrollment anticipated to be completed mid-2026. uniQure expects to present updated results from the Phase I/IIa clinical trial in the first half of 2027.

About the Phase I/IIa Clinical Program of AMT-260

GenTLE is a Phase I/IIa multi-center, open-label trial being conducted in the United States currently consisting of two dosing cohorts of up to six refractory MTLE patients each receiving a locally delivered, one-time intracerebral infusion of AMT-260. The study consists of an initial 12-month evaluation period followed by long-term follow-up for four years. The trial will evaluate the safety, tolerability and exploratory signs of efficacy of AMT-260 in individuals with refractory MTLE. Additional details are available on www.clinicaltrials.gov (NCT06063850).

About AMT-260

AMT-260 is a one-time administered, *in vivo* gene therapy candidate intended to reduce or eliminate seizures in people with refractory mesial temporal lobe epilepsy. AMT-260 is designed to locally deliver two engineered microRNAs to suppress the GRIK2 gene and the aberrant expression of GluK2, a subunit of a kainate glutamate receptor that is believed to trigger seizures in people with refractory MTLE.

About Refractory Mesial Temporal Lobe Epilepsy

Temporal lobe epilepsy is a chronic neurologic disorder and affects approximately 500,000 people in the United States, of which approximately 300,000 are inadequately treated through anti-seizure medications and are considered refractory. Approximately 80% of United States refractory temporal lobe epilepsy cases are mesial, which involves the medial (or internal) structures of the brain.

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 – a 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, 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 concerning: the advancement and intended effects of AMT-260, plans to complete enrollment in the second cohort of the Phase I/IIa clinical trial mid-2026, and plans to present updated results from the Phase I/IIa clinical trial in the first half of 2027. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, among others: risks associated with the clinical results and the development and timing of the Company's clinical programs; the risk that more patient data become available that results in different findings than that presented in preliminary or interim data; the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways and timing for regulatory

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, including AMT-260, in clinical trials; the Company's ability to obtain, maintain and protect 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 the Company's Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q, 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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