uniQure Presents Clinical Case Study of First Patient Dosed with AMT-260 in Refractory Mesial Temporal Lobe Epilepsy (MTLE)

May 29, 2025

~ No serious adverse events and 92% reduction in seizure frequency observed in the first trial participant through first five months of follow up ~

~ Data to be presented today at Epilepsy Therapies & Diagnostics Development Symposium (ETDD) ~

LEXINGTON, Mass. and AMSTERDAM, May 29, 2025 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the presentation of a clinical case study of the first participant dosed in the first cohort of its ongoing Phase I/IIa trial of an investigational gene therapy candidate, AMT-260, in patients with refractory MTLE. The data are being presented today, May 29, 2025 by Firas Taha, M.D., Medical Director, Clinical Development at uniQure, at the Epilepsy Therapies & Diagnostics Development Symposium in Leesburg, Virginia.

The first participant in the GenTLE clinical trial has been followed for five months post-administration of AMT-260. Prior to treatment, the participant experienced an average of seven seizures a month, including five seizures in the 30-day period immediately prior to dosing. Since receiving AMT-260, the participant has reported two seizures during the five-month follow-up period, with no seizures reported during the last 60 days as of April 17th (cutoff date), the last date data were obtained on this participant for presentation in the ETDD symposium case study and no reported serious adverse events as of the cutoff date.

“These early results from the first trial participant are very encouraging and support our belief that AMT-260 has the potential to be a valuable treatment alternative for people living with drug-resistant mesial temporal lobe epilepsy,” stated Walid Abi-Saab, M.D., chief medical officer of uniQure. “While additional follow-up on this first trial participant and data from additional participants in the trial are needed, the reduction in seizure frequency and tolerability in this first trial participant as of the cutoff date offer a compelling early signal of the potential impact of AMT-260 that warrants continued investigation. There remains a high unmet need for safer, more effective treatment options for people with drug-resistant MTLE and we’re eager to continue evaluating AMT-260 in additional trial participants.”

AMT-260 is a one-time administered, *in vivo* gene therapy candidate intended to reduce or eliminate seizures in people with drug-resistant 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.

GenTLE is a Phase I/IIa multi-center, open-label trial being conducted in the U.S. to evaluate the safety, tolerability and exploratory signs of efficacy of AMT-260 in individuals with refractory MTLE. The study is comprised of two dose cohorts of six patients each. The study is actively screening additional patients throughout 12 sites, with additional sites expected to be activated by the end of 2025. Additional details are available on www.clinicaltrials.gov (NCT06063850).

About Refractory Mesial Temporal Lobe Epilepsy

Temporal lobe epilepsy is a chronic neurologic disorder and is the most common form of focal epilepsy with more than 600,000 individuals suffering from the disorder in the United States. Approximately 80% of all temporal lobe epilepsy cases are mesial, which involves the medial (or internal) structures of the brain. The majority of MTLE cases are refractory to anti-seizure medications, which severely limits treatment options.

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. Other than statements of historical fact, all statements contained in this press release are forward-looking statements, including, without limitation, statements about the design, advancement and intended effects of AMT-260, our belief that AMT-260 has the potential to be a valuable treatment alternative for people living with drug-resistant mesial temporal lobe epilepsy, our goals for site expansion and patient enrollment for AMT-260 and other statements identified by the terms “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 not promises or guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control. Investors are cautioned that any forward-looking statements, including updates regarding the future development of gene therapy candidates, including AMT-260 for drug-resistant mesial temporal lobe epilepsy, are not guarantees of future performance or results and involve substantial risks and uncertainties. Actual results, developments and events may differ materially from those in the forward-looking statements as a result of various risk factors including: risks associated with the clinical results and the development and timing of our clinical programs; our interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to approval, if any; our effectiveness in managing current and future clinical trials and regulatory processes; our ability to demonstrate the therapeutic benefits of our gene therapy candidates, including AMT-260, in clinical trials; our ability to continue to build and maintain our infrastructure and personnel needed to achieve our goals; the continued development and acceptance of gene therapies; our ability to obtain, maintain and protect our intellectual property,

our ability to fund our operations and to raise additional capital as needed; and other risks as may be detailed from time to time under the heading "Risk Factors" in our periodic filings with the U.S. Securities & Exchange Commission (SEC), including our Annual Report on Form 10-K filed with the SEC on February 27, 2025, our Quarterly Report on Form 10-Q filed with the SEC on May 9, 2025, and in other filings that we make 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. The information contained in this press release is as of the date of this press release and we assume no obligation to update the forward-looking statements contained in this release as the result of new information or future events or developments.

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