uniQure Announces Dosing of First Patient in GenTLE Phase I/IIa Clinical Trial of AMT-260 for the Treatment of Refractory Mesial Temporal Lobe Epilepsy

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LEXINGTON, Mass. and AMSTERDAM, Nov. 21, 2024 (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 first patient has been dosed in the GenTLE Phase I/IIa clinical trial of AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE).

"The dosing of the first patient in our Phase I/II trial in temporal lobe epilepsy represents an important milestone for uniQure and our third clinical trial initiation over the past six months," stated Walid Abi-Saab, M.D., chief medical officer of uniQure. "Nearly one-third of people experiencing focal onset seizures do not respond to currently available treatments and are left with limited therapeutic options. Our investigational agent, AMT-260, which is a one-time administration, has the potential to be a transformative treatment option for these patients. We continue to actively screen patients for the trial and look forward to providing program updates in the new year."

AMT-260 consists of an AAV9 vector that locally delivers two engineered miRNAs designed to reduce the expression of GluK2 protein subunits, a subtype of glutamate receptor that is believed to be aberrantly expressed in the hippocampus of patients with refractory MTLE and believed to trigger their seizure activity. In preclinical animal studies, AMT-260 reduced the number of seizures per day in a dose-dependent manner. AMT-260 also reduced the expression of GluK2 mRNA and protein in the hippocampus of epileptic mice and from resected hippocampal slices from patients 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 two doses of AMT-260 in individuals with refractory MTLE. The study comprises two dose cohorts of six patients each. The study is actively recruiting though 10 sites with an additional two sites expected to be activated by the end of 2024. 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's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our 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. We are now leveraging our modular and validated technology and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (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 AMT-260's potential to be a transformative treatment option for these patients with MTLE; the potential efficacy profile of AMT-260 through one-time administration with the ability to reduce the frequency of seizures in MTLE patients; the Company's plans to announce additional updates on trial enrollment; the design of the AMT-260 Phase I/IIa clinical trial and plans to activate additional study sites. 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 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, its Quarterly Reports on Form 10-Q filed May 7, 2024, August 1, 2024 and 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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