uniQure Announces FDA Clearance of Investigational New Drug Application for AMT-260 Gene Therapy for Refractory Mesial Temporal Lobe Epilepsy

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Clinical trial initiation expected in the fourth quarter of 2023

LEXINGTON, Mass. and AMSTERDAM, Sept. 05, 2023 (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 U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for AMT-260, the Company's gene therapy candidate for refractory mesial temporal lobe epilepsy (MTLE). AMT-260 comprises an AAV9 vector that locally delivers two engineered miRNAs designed to degrade the GRIK2 gene and suppress the aberrant expression of glutamate receptor subtype GLUK2 that is believed to trigger seizures in patients with refractory MTLE.

"The clearance of the IND for AMT-260 is an important achievement in advancing our pipeline and is our next program to enter clinical development in an area of high unmet medical need," stated Walid Abi-Saab, chief medical officer of uniQure. "There are few treatment options for patients who have refectory MTLE, and we are pleased to soon begin the clinical investigation of this one-time administered gene therapy approach as a potential new treatment."

The first-in-human Phase I/IIa clinical trial will be conducted in the United States and consist of two parts. The first part is a multicenter, open-label trial with two dosing cohorts of six patients each to assess safety, tolerability, and first signs for efficacy of AMT-260 in patients with refractory MTLE. The second part is expected to be a randomized, controlled trial to generate proof of concept (POC) data. The clinical trial is expected to begin patient screening in the fourth quarter of 2023.

About AMT-260
AMT-260 is an AAV9 gene therapy product that locally delivers miRNA silencing technology to target the GRIK2 gene and suppress aberrantly expressed GluK2 containing kainate receptors. The therapeutic goal is to lower the expression of GluK2 containing kainate receptors which are believed to trigger epilepsy when aberrantly expressed in the epileptic hippocampus. AMT-260 represents a novel potential one-time administered approach to treating refractory MTLE.

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. 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 – represents 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. These forward-looking statements include, but are not limited to, statements regarding the timing of patient enrollment in the Company's open-label U.S. Phase I/IIa trial for refractory MTLE and the scope of treatment options for patients who have refractory MTLE. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of financial and geopolitical events on our Company and the wider economy and health care system, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 27, 2023 and the Quarterly Report on Form 10-Q filed August 1, 2023. 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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