uniQure Highlights Therapeutic Potential of AMT-260 in Refractory Temporal Lobe Epilepsy (rTLE) at Virtual Research & Development Event

November 29, 2022

~ IND Submission and Clinical Development Expected to Begin in 2023 ~

~ Advancements in Manufacturing and Technology Platform Also Highlighted ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Nov. 29, 2022 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, hosted a virtual investor event focused on AMT-260, an AAV gene therapy for refractory temporal lobe epilepsy (rTLE) and other focal epilepsies. The event featured a presentation from rTLE clinical expert Ellen Bubrick, MD, associate chair of Neurology at Harvard Medical School and director of the Epilepsy Surgery Program at Brigham and Women's Hospital in Boston, MA on the unmet medical need of patients with rTLE. The presentation highlighted preclinical data that supports the safety and tolerability of AMT-260 and plans for its clinical development, as well as uniQure's miQURE™ and linQURE™technology platforms that allow the Company to use miRNAs to safely reduce the expression of genes in the brain. The Company also highlighted its progress in developing a commercial-scale AAV manufacturing platform. A replay of the investor event is available here.

"One week after the landmark FDA approval of the uniQure-developed world's first gene therapy for hemophilia B, we are pleased to share the progress that we have made in developing AMT-260, a first-in-class gene therapy for temporal lobe epilepsy," stated Ricardo Dolmetsch, Ph.D., president of research & development at uniQure. "Today's event presented the preclinical data and the innovative technologies that we hope will allow us to develop AMT-260 and other gene therapies that can locally regulate neuronal activity for diseases of the central nervous system. We look forward to continuing to work toward submitting an investigational new drug application for AMT-260 and advancing it into the clinic in 2023."

AMT-260 in refractory Temporal Lobe Epilepsy (rTLE)

AMT-260 is an AAV gene therapy that uses an AAV9 vector and combines uniQure's miQURE and linQURE technologies to deliver multiple miRNAs that reduce the expression of the GRIK2 gene. The GRIK2 gene encodes a subunit of the kainate glutamate receptor, which plays a key role in neuronal excitability and in the pathogenesis of rTLE. It is the first example of uniQure's broader CNS gene therapy platform that uses local delivery of an AAV to reduce the expression of neurotransmitter receptors and control the excitability of neurons. Pathological activation of neurons underlies diseases such as epilepsies, movement disorders and pain syndromes. AMT-260 is being developed initially for rTLE, but the platform is applicable to other focal epilepsies.

In preclinical mouse studies, AMT-260 has shown a dose-dependent increase in the expression of miRNAs in the mouse hippocampus as well as decreased expression of GRIK2 gene. AMT-260 has been shown to inhibit epileptic activity and improve health in a mouse model of TLE . The Company has initiated an IND-enabling GLP toxicology study in non-human primates and to date, AMT-260 has been well tolerated and shown no relevant safety findings. uniQure is planning to conduct a Phase I/II study of AMT-260 starting in 2023.

Advances in Manufacturing Platform Technology

uniQure also provided an update on advances in its industry-leading gene therapy manufacturing platform and on innovations the Company has made to improve quality, decrease costs, and improve speed of manufacturing AAV therapies.

"We continue to capitalize on the knowledge of our platform, which is used to manufacture our recently-approved gene therapy for hemophilia B, while improving it for our new programs, enabling us to save precious time during development," stated Pierre Caloz, chief operating officer at uniQure. "The recent development of our manufacturing modules allows us to achieve cost reductions typically seen in more mature biotech domains. We are very pleased that our platform may allow us to advance the exciting promise of AAV gene therapy into larger patient populations."

The full virtual research and development event program was webcast live under the Investors section of uniQure's website at www.uniQure.com. A replay of the webcast will be available at uniQure's website for 45 days following the event.

About Temporal Lobe Epilepsy
Temporal lobe epilepsy (TLE) is epilepsy that starts in the temporal lobe area of the brain. There are two temporal lobes, one on each side of the head located behind the temples. TLE is the most common localized, also called "focal," type of epilepsy. About 60% of people with focal epilepsy have TLE.

About 80% of all temporal lobe seizures start in the mesial temporal lobe, with seizures often starting in or near the hippocampus which controls memory and learning. Mesial temporal lobe epilepsy is the most common form of epilepsy. In neocortical or lateral TLE, seizures start in the outer section of the temporal lobe. This type of TLE is very rare and mostly due to a genetic cause or lesions such as a tumor, birth defect, blood vessel abnormality or other abnormalities in the temporal lobe.

Temporal lobe epilepsy affects approximately 1.3 million people in the U.S. alone, of which approximately 800,000 patients are unable to adequately control acute seizures with currently approved anti-epileptic therapies. Patients with refractory temporal lobe epilepsy (rTLE) experience increased morbidity, excess mortality, and poor quality of life.

About uniQure
uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. We are leveraging our modular and validated technology platform to advance a pipeline of proprietary gene therapies to treat patients with hemophilia B, Huntington's disease, refractory temporal

lobe epilepsy, Fabry disease, and other 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," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "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, the achievement of any of our planned near term or other milestones such as the submission of an investigational new drug application for AMT-260 or advancing it into the clinic in 2023, and the ability of our manufacturing platform to help us more effectively develop our pipeline products. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our and our collaboration activities, product development activities, corporate reorganizations and strategic shifts, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's Annual Report on Form 10-K filed on February 25, 2022 and Quarterly Report on Form 10-Q filed on November 2, 2022. Given these risks, uncertainties, and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Chiara Russo
Direct: 617-306-9137
Mobile: 617-306-9137
c.russo@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com