uniQure Announces FDA Clearance of Investigational New Drug Application for AMT-191 Gene Therapy for Fabry Disease

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Patient enrollment expected to begin in first half of 2024

LEXINGTON, Mass. and AMSTERDAM, Nov. 29, 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-191, the Company's gene therapy candidate for Fabry disease. AMT-191 comprises an AAV5 vector that delivers an α-galactosidase A (GLA) transgene designed to target the liver and produce the deficient GLA protein.

"The clearance of the IND for AMT-191 represents a key milestone for the company, with four programs now in clinical phase," stated Walid Abi-Saab, M.D., chief medical officer of uniQure. "AMT-191 has the potential to be a differentiated gene therapy for the one-time treatment of Fabry disease, incorporating a proprietary promoter and leveraging our validated AAV5 technology comprised within HEMGENIX®, an approved liver-directed gene therapy for the treatment of hemophilia B developed by uniQure. We have designed the Phase I/II study to provide dose-ranging biomarker data as rapidly and cost-effectively as possible, and we look forward to enrolling our first patient in the first half of 2024."

The first-in-human Phase I/IIa clinical trial will be conducted in the United States. The multicenter, open-label trial consists of two dose-escalating cohorts of three patients each to assess safety, tolerability, and efficacy of AMT-191 in patients with Fabry disease.

About AMT-191
AMT-191 is an AAV5 gene therapy product that delivers a GLA transgene designed to target the liver to produce GLA protein. In patients with Fabry disease, a pathogenic variant in the GLA gene leads to GLA enzyme deficiency which results in a progressive accumulation of lipids in multiple cell types creating a multi-system disorder. AMT-191 represents a novel potential one-time administered approach to treating Fabry disease.

About Fabry Disease
Fabry disease is an X-linked genetic disorder resulting from a deficiency of GLA. The prevalence is estimated to be between 1 in 40,000 and 1 in 117,000 individuals[1]. The current standard of care for Fabry disease is bi-weekly infusions of enzyme replacement therapy, a treatment with limited effectiveness in many patients due to poor cross-correction, with inefficient clearance of substrates in the target organs, in particular the kidney and the heart.

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. These forward-looking statements include, but are not limited to, statements regarding the timing of patient dosing in the Company's open-label U.S. Phase I/IIa trial for Fabry disease and the scope of treatment options for patients who have Fabry disease. 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 the Company and the wider economy and health care system, the Company's ability to raise additional capital to support late stage development of the Company's clinical program(s) if supported by future data, the Company's 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 November 7, 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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[1] Vardarli, I., et al. Diagnosis and Screening of Patients with Fabry Disease. Ther Clin Risk Manag. 2020; 16: 551-558

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