uniQure Announces Completion of Enrollment in the First Cohort and Favorable Recommendation from the Independent Data Monitoring Committee for its Phase I/IIa Clinical Trial of AMT-191 for the Treatment of Fabry Disease

February 3, 2025

~ Independent Data Monitoring Committee recommends proceeding with dosing of second cohort after planned safety assessment ~

~ Company expects to initiate enrollment of second dose cohort in the first quarter of 2025 ~

LEXINGTON, Mass. and AMSTERDAM, Feb. 03, 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 completion of enrollment in the first cohort of the Phase I/IIa trial of AMT-191, an investigational gene therapy for the treatment of Fabry disease. Additionally, the Independent Data Monitoring Committee (IDMC) reviewed safety data from the initial two patients enrolled in the first cohort. The IDMC's review identified no significant safety concerns and recommended proceeding with enrollment in the second cohort.

"Fabry is a debilitating disease that continues to represent a significant unmet medical need," stated Walid Abi-Saab, M.D., chief medical officer of uniQure. "We are encouraged by the initial pharmacodynamics, biomarkers and safety profile observed to date for AMT-191 as well as the positive outcome of the IDMC review. This strengthens our confidence in the potential of AMT-191 to make a meaningful difference in the lives of patients with Fabry disease. We look forward to advancing to the second cohort in this important clinical program."

AMT-191 is an investigational AAV5-based gene therapy that uses a proprietary, highly potent promoter to deliver a galactosidase alpha (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 α-galactosidase A (aGAL-A) enzyme deficiency, which in turn results in a progressive accumulation of lipids in multiple cell types, including kidney and heart cells, eventually resulting in a multi-system disorder. AMT-191 may offer a novel one-time intravenously administered approach to treating Fabry disease.

About the Phase I/IIa Clinical Program of AMT-191

The Phase I/IIa clinical trial of AMT-191 is a multi-center, open-label trial being conducted in the United States consisting of two dosing cohorts of up to six adult male patients each receiving an intravenous infusion of AMT-191. Patients will continue to receive their regular enzyme replacement therapy until the criteria for withdrawal is met and will be followed for a period of 24 months. The trial will explore the safety, tolerability, and early signs of efficacy by measuring the expression of lysosomal enzyme aGLA-A. Additional details are available on www.clinicaltrials.gov (NCT06270316).

AMT-162 has been granted both Orphan Drug status and Fast Track designation by the U.S. Food and Drug Administration.

About Fabry Disease

Fabry disease is an X-linked genetic disorder resulting from a deficiency of GLA. Based on a 2020 study published in the Journal of Therapeutics and Clinical Risk Management, the prevalence is estimated to be between one in 40,000 and one in 117,000 individuals. 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 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. 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 the Company's plans to initiate enrollment of the second dose cohort of the AMT-191 study in the first quarter of 2025; the potential of AMT-191 to make a meaningful difference in the lives of patients and as a novel one-time intravenously administered approach to treating Fabry disease. 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; risks associated with the implementation of the Company's restructuring; 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 Report on Form 10-Q filed 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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