uniQure Announces Dosing of First Patient in Phase I/IIa Clinical Trial of AMT-191 for the Treatment of Fabry Disease

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LEXINGTON, Mass. and AMSTERDAM, Aug. 15, 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 a Phase I/IIa clinical trial of AMT-191 for the treatment of Fabry disease, a rare, inherited genetic disease. The Phase I/IIa study is a multi-center, open-label trial being conducted in the United States with two dose-escalating cohorts assessing the safety, tolerability and early signs of efficacy of AMT-191 in individuals with Fabry disease.

“We are very pleased to begin patient dosing for AMT-191 in Fabry disease, marking a significant milestone in this year’s goal to advance three new gene therapy candidates into clinical studies,” stated Walid Abi-Saab, M.D., chief medical officer of uniQure. “AMT-191 utilizes the same AAV delivery technology incorporated in HEMGENIX®, which has an extensive, long-term safety profile and demonstrated effectiveness in patients with preexisting neutralizing antibodies to the AAV capsid. Our trial is designed to capture well-established endpoints in Fabry disease and to rapidly generate clinical proof-of-concept data for AMT-191 with a differentiated product profile relative to other Fabry programs in clinical development.”

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 potential one-time intravenously administered approach to treating Fabry disease.

The Phase I/IIa clinical trial of AMT-191 will be conducted in the United States. The multicenter, open-label trial consists of two cohorts with up to six adult male patients each: a low-dose cohort of $6x10^{13}$ gc/kg and a high-dose cohort of $3x10^{14}$ gc/kg delivered through a one-time intravenous infusion. 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).

“This achievement marks an exciting period for the company as we advance additional programs into clinical trials this year,” stated Matt Kapusta, chief executive officer of uniQure. “Building on our momentum, we are focused on multiple, exciting catalysts across our pipeline, including engaging with the FDA to pursue an expedited clinical pathway for AMT-130 in Huntington’s disease and the initiation of new clinical studies in temporal lobe epilepsy and SOD1-ALS. With a strong balance sheet and runway extended through the end of 2027 from multiple cost reduction initiatives, we believe we are in an excellent position to execute on key value-creating milestones.”

About Fabry Disease

Fabry disease is an Xlinked- 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 timing of patient dosing and the availability of initial clinical and proof-of-concept data in the Company’s open-label U.S. Phase I/IIa trial for Fabry disease; the trial design and the differentiated profile of AMT-191 relative to other Fabry programs currently in clinical development. 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 plans; the Company’s ability to continue to build and maintain the company infrastructure and personnel needed to achieve its goals following planned workforce reductions; 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 and August 1, 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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