uniQure Announces FDA Breakthrough Therapy Designation Granted to AMT-130 for the Treatment of Huntington's Disease

April 17, 2025

~ Breakthrough Therapy designation based on clinical evidence from Phase I/II trials showing meaningful slowing of disease progression ~

~ Additional regulatory update and guidance on the Biologics License Application submission expected in the second quarter of 2025 ~

LEXINGTON, Mass. and AMSTERDAM, April 17, 2025 (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 granted Breakthrough Therapy designation to AMT-130 for the treatment of Huntington's disease, a rare, inherited neurodegenerative disorder for which there are currently no disease-modifying therapies available. This designation is in addition to Regenerative Medicine Advanced Therapy (RMAT) designation, Orphan Drug designation and Fast Track designation, all previously granted by the FDA to AMT-130.

"Receiving Breakthrough Therapy designation underscores both the urgent need for effective treatments for Huntington's disease and the encouraging interim data demonstrating that AMT-130 has the potential to slow disease progression," said Walid Abi-Saab, M.D., chief medical officer of uniQure. "It's a powerful recognition of the promise of AMT-130 and the important progress we've made. We deeply value the FDA's continued commitment to advancing innovative gene therapies for patients with critical unmet needs, and we look forward to working closely with the agency to bring AMT-130 to the Huntington's disease patient community as quickly as possible."

The Breakthrough Therapy designation is supported by clinical data from the ongoing Phase I/II trials of AMT-130 for the treatment of Huntington's disease. In July 2024, uniQure presented interim data at 24 months that showed dose-dependent slowing of disease progression based on the cUHDRS of treated patients compared to a propensity-weighted natural history. To date, a total of 45 patients have received AMT-130.

Breakthrough Therapy designation is intended to expedite the development and review of investigational therapeutic candidates that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). In general, the preliminary clinical evidence should show a clear advantage over available therapy. A drug that receives Breakthrough Therapy designation is eligible for all Fast Track designation features, intensive guidance on an efficient drug development program, and FDA commitment involving senior managers[1].

About Huntington's Disease

Huntington's disease is a rare, inherited neurodegenerative disorder that leads to motor symptoms including chorea, behavioral abnormalities and cognitive decline resulting in progressive physical and mental deterioration. The disease is an autosomal dominant condition with a disease-causing CAG repeat expansion in the first exon of the huntingtin gene that leads to the production and aggregation of abnormal protein in the brain. According to 2021 study in Neuroepidemiology, approximately 70,000 people have been diagnosed with Huntington's disease in the U.S. and Europe, with hundreds of thousands of others at risk of inheriting the disease. Despite the clear etiology of Huntington's disease, there are currently no approved therapies to delay the onset or to slow the disease's progression.

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 within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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 and the negatives of those terms. Forward-looking statements are based on management's beliefs and assumptions and on information available to management as of the date of this press release. Examples of these forward-looking statements include, but are not limited to, statements concerning: the Company's plans for further interactions with the FDA to discuss the requirements for its planned BLA submission for AMT-130; the Company's ability to utilize an accelerated pathway to progress AMT-130 through regulatory approval; the Company's plans to announce additional interim data and regulatory updates from its ongoing Phase I/II clinical studies of AMT-130, along with an initial safety update on the third cohort of the AMT-130 study and other program updates ; the potential clinical and functional effects of AMT-130; and the Company's plans to continue clinical development of AMT-130. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, among others: 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 regulatory approval; 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 with the SEC on February 27, 2025, 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, except as required by law, 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] Guidance for Industry Expedited Programs for Serious Conditions - Drugs and Biologics, May 2014