uniQure Announces Successful UK MHRA Pre-Submission Meeting for AMT-130 in Huntington's Disease

April 30, 2026

~ Submission of a UK Marketing Authorization Application for AMT-130 is expected in the third quarter of 2026 ~

~ Type B Meeting with U.S. FDA granted in second quarter; expect to discuss potential Phase III design and analysis plan for AMT-130 four-year data ~

~ uniQure actively pursuing additional ex-US regulatory pathways to support potential registration of AMT-130 in international markets ~

LEXINGTON, Mass. and AMSTERDAM, April 30, 2026 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the Company held a Pre-Submission Meeting with the United Kingdom's (UK) Medicines and Healthcare products Regulatory Agency (MHRA) and plans to submit a Marketing Authorization Application (MAA) for AMT-130 for the treatment of Huntington's disease in the third quarter of 2026.

"We are encouraged by the constructive feedback from the MHRA as we continue to work towards a regulatory submission for AMT-130 in Huntington's disease, which we now expect to submit in the third quarter of this year," said Matt Kapusta, chief executive officer of uniQure. "This is an important milestone for the Huntington's disease community and we remain committed to working closely with regulators globally, with the goal of bringing this potentially transformative therapy to patients in the UK and internationally."

During the meeting, the Company and MHRA discussed the data package and manufacturing requirements to support a MAA submission for AMT-130. Following these discussions, the Company expects to submit a MAA based on the three-year analysis from the ongoing U.S. and European Phase I/II clinical trials. At year three, these data showed a statistically significant 75% slowing of disease progression at the high dose as measured by the composite Unified Huntington's Disease Rating Scale compared to a propensity score-matched external control (p=.003) and was generally well-tolerated, with a manageable safety profile.

In addition to the continued engagement with the MHRA, the Company has been granted a Type B meeting with the U.S. Food and Drug Administration in the second quarter of 2026. The Company expects to discuss key elements of a potential Phase III trial design and to receive feedback on the proposed statistical analysis plan for the four-year analysis expected in the third quarter of 2026. The Company is also actively pursuing additional regulatory pathways in international markets for potential registration of AMT-130 and expects to provide further updates in the second half of 2026.

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 – 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. uniQure is now advancing a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, 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 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: plans to submit a Marketing Authorization Application in the third quarter of 2026 for AMT-130 based on the three-year analysis from the ongoing Phase I/II clinical trials; plans to meet with the U.S. Food and Drug Administration (FDA) in the second quarter of 2026 to discuss a potential Phase III trial design for AMT-130 and a statistical analysis plan for a four-year analysis of AMT-130 expected in the third quarter of 2026; plans to work with regulators globally to potentially bring AMT-130 to patients; plans to provide future updates regarding the Company's interactions with regulatory authorities; and the potential benefits 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 related to the Company's Phase I/II clinical trials of AMT-130, including the risk that such trials will be unable to demonstrate data sufficient to support further clinical development or regulatory approval; the risk that more patient data become available that results in a different interpretation then the one derived from topline data; risks related to the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to regulatory approval; the risk that the Company will be unable to align with the FDA, MHRA, or other regulatory authorities on an approval pathway for its gene therapy candidates, including AMT-130; whether the measurements that the Company is evaluating are viewed as robust and sensitive measurements of disease progression; whether RMAT designation, Breakthrough Therapy designation, or any accelerated pathway, if granted, will lead to regulatory approval; the Company's ability to conduct and fund a Phase III or confirmatory study for AMT-130; the Company's ability to continue to build and maintain the infrastructure and personnel needed to achieve its goals; the Company's effectiveness in managing current and future clinical trials and regulatory processes; the Company's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; the continued development and acceptance of gene therapies; the Company's ability to obtain, maintain and protect its intellectual property; and the Company's ability to fund its operations and to raise additional capital

as needed and on acceptable terms. 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 Reports on Form 10-K , its Quarterly Reports on Form 10-Q 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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