uniQure Provides Regulatory Update on AMT-130 for Huntington's Disease

December 4, 2025

LEXINGTON, Mass. and AMSTERDAM, Dec. 04, 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 company received final meeting minutes from the U.S. Food and Drug Administration (FDA) regarding a pre-Biologics License Application (BLA) meeting held on October 29, 2025 to discuss the application for AMT-130, an investigational gene therapy for Huntington's disease (HD).

In the final meeting minutes, and consistent with uniQure's November 3, 2025 press release, the FDA conveyed that data submitted from the Phase I/II studies of AMT-130 are currently unlikely to provide the primary evidence to support a BLA submission. uniQure is carefully evaluating the feedback and plans to urgently request a follow-up meeting with the FDA to take place in the first quarter of 2026.

"We are committed to collaborating with the FDA to advance AMT-130 to patients and their families as rapidly as possible," said Matt Kapusta, chief executive officer at uniQure. "The support we have seen these last weeks from the Huntington's disease community, including patients, families, caregivers, clinicians and advocates reinforces the urgency of the unmet need in Huntington's disease."

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 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 to request a follow-up meeting with the FDA to take place in the first quarter of 2026 and to collaborate with the FDA to advance AMT-130 to patients and families, and the timing and outcome of regulatory interactions with respect to the AMT-130 program. 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 the FDA ultimately concludes that such trials are not adequate and well-controlled to provide the primary evidence to support a BLA; the risk that more patient data become available that results in a different interpretation then the one derived from the 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; 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 if needed; 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 Report on Form 10-K filed with the SEC on February 27, 2025, its Quarterly Reports on Form 10-Q filed with the SEC on May 9, 2025, and July 29, 2025, and November 10, 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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