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

March 2, 2026

LEXINGTON, Mass. and AMSTERDAM, March 02, 2026 (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 Type A meeting held on January 30, 2026 to discuss AMT-130, an investigational gene therapy for Huntington's disease (HD).

The FDA stated that it cannot agree that data from the Phase I/II studies, compared to an external control, are sufficient to provide the primary evidence of effectiveness required to support a marketing application for AMT-130. The FDA strongly recommended uniQure conduct a prospective, randomized, double-blind, sham surgery-controlled study. uniQure intends to continue engaging with the FDA regarding Phase III development considerations and plans to request a Type B meeting in the second quarter of 2026 to further discuss potential study design approaches.

"While we did not reach alignment on a submission pathway based on the Phase I/II data, we believe the totality and durability of our data warrant continued substantive dialogue regarding how the FDA's stated commitment to regulatory flexibility may be appropriately applied in this setting," said Matt Kapusta, chief executive officer at uniQure. "We remain committed to engaging with the FDA to determine a clear, scientifically grounded, and efficient path forward for AMT-130. We are deeply grateful for the resilience and support of the Huntington's disease community and remain committed to standing with patients and their families as we advance this potentially transformative therapy for a community in need."

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: plans to continue engaging with the FDA regarding Phase III development considerations for AMT-130; plans to request a Type B meeting with the FDA to take place in the second quarter of 2026; and the potential benefit 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 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; the risk that we will be unable to align with the FDA or other regulatory authorities on an approval pathway for our 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.

uniQure Contacts:

FOR INVESTORS:

Chiara Russo
Direct: 781-491-4371
Mobile: 617-306-9137
c.russo@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com

uniQure