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

November 3, 2025

LEXINGTON, Mass. and AMSTERDAM, Nov. 03, 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 it received feedback from the U.S. Food and Drug Administration (FDA) during a recent pre-Biologics License Application (BLA) meeting regarding AMT-130, an investigational gene therapy for Huntington's disease (HD).

Though final meeting minutes have not yet been received, based on the discussions at the meeting, uniQure believes that the FDA currently no longer agrees that data from the Phase I/II studies of AMT-130 in comparison to an external control, as per the prespecified protocols and statistical analysis plans shared with the FDA in advance of the analyses, may be adequate to provide the primary evidence in support of a BLA submission. This is a key shift from prior communications with the FDA in multiple Type B meetings over the past year. Consequently, the timing of the BLA submission for AMT-130 is now unclear.

uniQure expects to receive final minutes within 30 days of the meeting and plans to urgently interact with the FDA to find a path forward for the timely accelerated approval of AMT-130.

The FDA granted AMT-130 Breakthrough Therapy designation based upon data from the Phase I/II studies compared to external controls in April 2025 and Regenerative Medicines Advanced Therapy (RMAT) designation in May 2024.

"We are surprised by the FDA's feedback at the recent pre-BLA meeting, which is a drastic change from the guidance the FDA provided in November 2024 that data from the ongoing Phase I/II studies, compared to a natural history external control, may serve as the primary basis for a BLA submission under the Accelerated Approval pathway," said Matt Kapusta, chief executive officer at uniQure. "This news is unexpected, and we are truly disappointed for people living with HD, who have no disease-modifying treatment options for this devastating disease. We strongly believe that AMT-130 has the potential to bring substantial benefit to patients, and we remain fully committed to working with the FDA to determine the best path forward to rapidly bring AMT-130 to patients and their families in the U.S."

In addition to continuing to partner with the FDA on progressing AMT-130 for the treatment of Huntington's disease, uniQure plans in parallel to progress discussions with other regulatory agencies, including in the European Union and United Kingdom.

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 progress AMT-130 in the U.S., including the Company's plans to interact with the FDA to find a path forward for the timely accelerated approval of AMT-130 and rapidly bring AMT-130 to patients and their families in the U.S.; the timing and outcome of regulatory interactions with respect to the AMT-130 program, including the Company's plans to progress discussions with other regulatory agencies, including in the European Union and United Kingdom, in parallel with regulatory discussions with the FDA; the receipt of final minutes from the pre-BLA meeting with the FDA within 30 days of the meeting; the timing of the Company's BLA submission for AMT-130; the Company's belief that the FDA no longer agrees that data from the Phase I/II studies of AMT-130 in comparison to an external control may be adequate to provide the primary evidence in support of a BLA submission; and the potential of AMT-130 to bring substantial benefit to patients. 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; 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 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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