uniQure Announces Plan for BLA Submission for AMT-130 in Huntington's Disease

June 17, 2026

~ 3-year analysis from the Phase I/II study can serve as the primary basis of a Biologics License Application for accelerated approval with FDA ~

~ Company intends to submit the BLA in third quarter of 2026 ~

LEXINGTON, Mass. and AMSTERDAM, June 17, 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, during a recent Type B meeting with the U.S. Food and Drug Administration (FDA), the FDA communicated that the 3-year analysis from the Phase I/II study would be acceptable as the primary basis of a Biologics License Application (BLA) for the accelerated approval of AMT-130 in Huntington's disease. In addition, the FDA seeks to align on the confirmatory study design prior to the BLA submission, including consideration of concurrent control on standard-of-care therapy instead of a sham procedure. FDA communicated that they would work as expeditiously as possible with uniQure on this effort. The Company is committed to conducting the confirmatory study without delay and expects to further align with the FDA on the details of such a study prior to BLA submission. The Company intends to submit the BLA in the third quarter of 2026.

"Today's announcement reflects the outcome we have worked toward throughout our continued regulatory engagement with FDA, and we are deeply grateful for FDA's genuine commitment to addressing the unmet need of Americans living with Huntington's disease," said Matt Kapusta, chief executive officer at uniQure. "The FDA has agreed that our current clinical data can support a near-term BLA submission and has committed to work expeditiously with us to align on the design of the required confirmatory study. The consistency and strength of the clinical data generated to date give us great confidence in the product's potential to make a meaningful difference for patients. We remain focused on bringing AMT-130 to patients and families as quickly and responsibly as possible in the US and globally."

The Company expects to receive final minutes within 30 days of the recent Type B meeting.

AMT-130 has been granted Regenerative Medicine Advanced Therapy (RMAT) designation by the FDA – the first RMAT designation for Huntington's disease – as well as Breakthrough Therapy designation and Fast Track designation.

About the Phase I/II Clinical Program of AMT-130

uniQure is conducting two multi-center, dose-escalating, Phase I/II clinical studies to explore the safety, tolerability, and exploratory efficacy signals of AMT-130 for the treatment of Huntington's disease. Based on interactions with the FDA, it was agreed that data from cohorts 1 and 2 in the Phase I/II studies could be compared to a propensity score-matched external control derived from the Enroll-HD natural history data set, under a prespecified statistical analysis plan, which may serve as the primary basis for a BLA submission.

In the U.S. study, a total of 26 patients with early manifest Huntington's disease were randomized to treatment (n=6 low dose; n=10 high dose) or an imitation (sham) procedure (n=10). Treated patients received a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). The study consists of a blinded 12-month core study period followed by unblinded long-term follow-up of treated patients for five years. An additional four control patients crossed over to treatment. The European open-label Phase 1b/2 study of AMT-130 enrolled 13 patients with early manifest Huntington's disease (n=6 low dose; n=7 high dose).

A third cohort enrolled an additional 12 patients across sites in the U.S. and EU. This cohort was randomized to explore both doses of AMT-130 in combination with immunosuppression, using the current, established stereotactic administration procedure.

A fourth U.S. based cohort enrolled six patients and is evaluating high-dose AMT-130 in patients with lower striatal volumes compared to those of patients enrolled in previous cohorts.

Additional details are available on www.clinicaltrials.gov (NCT05243017, NCT04120493)

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. Approximately 75,000 people have Huntington's disease in the U.S.[1], EU[2], and the UK[3], 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, 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 and Section 21E of the Exchange Act. 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 only as of the date of this report. Examples of these forward-looking statements include, but are not limited to, statements concerning: the Company's plans to conduct a confirmatory study, including the design, timing, endpoints and control arm of such study, and to align with FDA on such study prior to BLA submission, the potential use of a concurrent standard-of-care control arm rather than a sham procedure, and expectations regarding timing of BLA submission and receiving accelerated approval 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 continue to demonstrate data sufficient to support further clinical development or regulatory approval; the risk that the FDA ultimately concludes that the Phase I/II trial data are not sufficient to support a BLA or accelerated approval; the risk that more patient data become available that results in a different interpretation than the one derived from the year three data analyses; 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 any required confirmatory study for AMT-130; the Company's ability to successfully complete any required confirmatory study for AMT-130; the risk that accelerated approval, if granted, may be subject to post-approval requirements that are difficult or costly to satisfy; 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 March 2, 2026, its Quarterly Report on Form 10-Q filed with the SEC on May 5, 2026 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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[2] Medina A, et al. Mov Disord 2022;37(12):2327–2335

[3] Furby H, et al. Eur J Neurol 2022;29(8):2249–2257.