uniQure Announces Submission of Biologics License Application for Ifezuntirgene Inilparvovec (AMT-130) in Huntington's Disease

September 2, 2026

~ Company also announces the submission of a Marketing Authorisation Application for ifezuntirgene inilparvovec in the U.K. ~

~ Both submissions are supported by the three-year data analysis from the Phase I/II study, in which ifezuntirgene inilparvovec demonstrated a slowing of disease progression ~

LEXINGTON, Mass. and AMSTERDAM, Sept. 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 the submission of a Biologics License Application (BLA) to the United States (U.S.) Food and Drug Administration (FDA) for the accelerated approval of ifezuntirgene inilparvovec (AMT-130), an investigational gene therapy for the treatment of Huntington's disease. The Company also announced that its Marketing Authorisation Application (MAA) for ifezuntirgene inilparvovec has been submitted to the United Kingdom's (U.K.) Medicines and Healthcare products Regulatory Agency (MHRA).

"The submission of licensing applications for ifezuntirgene inilparvovec represents an important milestone for the Huntington's disease community," said Matt Kapusta, chief executive officer at uniQure. "We are grateful to the FDA for its leadership in advancing regulatory science to meet the urgency of this disease, and to the MHRA for its commitment to advancing rare disease treatments in the U.K. We look forward to working with both agencies as these applications progress."

The Company has requested priority review for the BLA. If granted, priority review would shorten the FDA review cycle to six months following the FDA's 60-day BLA filing review period.

The BLA and MAA are supported by the previously announced three-year data analysis from the Phase I/II clinical study of ifezuntirgene inilparvovec, compared to a propensity score-matched external control derived from the Enroll-HD natural history database. The Company intends to present a four-year data analysis from the ongoing Phase I/II clinical studies before the end of the current third quarter.

Ifezuntirgene inilparvovec is the first investigational therapy for Huntington's disease to have received Breakthrough Therapy and Regenerative Medicine Advanced Therapy (RMAT) designations from the FDA. Ifezuntirgene inilparvovec also holds Fast Track designation from the FDA.

About Ifezuntirgene Inilparvovec (AMT-130)

Ifezuntirgene inilparvovec is a novel gene therapy candidate for the treatment of Huntington's disease, which utilizes a proprietary, gene-silencing miQURE® platform and incorporates a miRNA, specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment. Treated patients receive a single administration through targeted, MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen).

About the Phase I/II Clinical Program of Ifezuntirgene Inilparvovec

uniQure is conducting two multi-center Phase I/II clinical studies evaluating the safety, tolerability, and efficacy of ifezuntirgene inilparvovec for the treatment of Huntington's disease.

The U.S. randomized study enrolled 26 patients who received either a single administration of ifezuntirgene inilparvovec (n=6 low dose; n=10 high dose) or a sham procedure (n=10); four control patients subsequently crossed over to treatment after approximately 12 months. The European open-label study enrolled 13 patients (n=6 low dose; n=7 high dose). A third cohort of 12 patients explored both doses in combination with immunosuppression, and a fourth cohort of six U.S. patients is evaluating the high dose in patients with lower striatal volumes compared to those of patietns 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. 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 press release. Examples of these forward-looking statements include, but are not limited to, statements concerning: the BLA and MAA review of ifezuntirgene inilparvovec for the treatment of Huntington's disease by the FDA and MHRA, respectively; the potential priority review, and timing associated therewith, of the BLA for ifezuntirgene inilparvovec; and plans to present a four-year analysis from the Phase I/II studies of ifezuntirgene inilparvovec by the end of the current third quarter. 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 ifezuntirgene inilparvovec, 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 regulatory authorities, including the FDA and MHRA, ultimately conclude that the Phase I/II trial data are not sufficient to support regulatory approval, including accelerated approval with respect to a BLA; the risk that additional patient data leads to a different interpretation than the one derived from the year three data analysis; risks related to the Company's interactions with regulatory authorities, including the FDA and MHRA, which may affect the initiation, timing and progress of clinical trials and pathways to regulatory approval; the risk that the FDA may not accept for filing the Company's BLA and the MHRA may not validate the Company's MAA for ifezuntirgene inilparvovec, or that such acceptance or validation is delayed or additional information is required to be provided by the Company; 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 ifezuntirgene inilparvovec; the Company's ability to successfully complete any required confirmatory study for ifezuntirgene inilparvovec; 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 the Company's Annual Reports on Form 10-K and 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

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