uniQure Announces FDA Acceptance and Priority Review of the Biologics License Application for ifezuntirgene inilparvovec for the Treatment of Huntington's Disease

November 2, 2026

~ FDA sets PDUFA target action date of May 1, 2027 ~

~ If approved, ifezuntirgene inilparvovec will be the world's first disease-modifying treatment for Huntington's disease ~

LEXINGTON, Mass. and AMSTERDAM, Nov. 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 U.S. Food and Drug Administration (FDA) has accepted for review the Biologics License Application (BLA) for ifezuntirgene inilparvovec, an investigational gene therapy for the treatment of Huntington's disease submitted under the Accelerated Approval pathway. The application has been granted Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of May 1, 2027.

In its acceptance letter, the FDA stated that they are tentatively planning to discuss this application at an Advisory Committee meeting. The Company will be notified once the date and timelines have been finalized.

"The FDA's acceptance of our BLA for review marks an important milestone for the Huntington's disease community, which has waited generations for a treatment that changes the course of this devastating disease," stated Matt Kapusta, chief executive officer of uniQure. "Backed by compelling data from our Phase I/II studies, we believe ifezuntirgene inilparvovec has the potential to become the first disease modifying treatment for Huntington's disease. With the FDA's priority review underway, we will work in conjunction with the Agency, clinicians, and the Huntington's community to support the review process. The confirmatory trial remains a key priority, with screening anticipated to begin by year end and the study is expected to be well underway by the time of potential approval."

The BLA is supported by the previously announced 36-month 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, as previously announced on September 24, 2025.

The Company and FDA have aligned on key elements of the confirmatory study design. The study is designed as a randomized, standard-of-care trial enrolling approximately 200 patients in a 2:1 ratio of ifezuntirgene inilparvovec to standard of care, with Total Functional Capacity (TFC) as the primary endpoint at 36 months. The Company plans to initiate patient screening in the U.S. by the end of 2026.

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.

In total, the Phase I/II clinical studies have dosed 51 patients with ifezuntirgene inilparvovec. 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 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," "expect," "intend," "look forward to," "may," "plan," "potential," "seek," "should," "will" 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 beliefs related to the review and approval of its biologics license application ("BLA") for ifezuntirgene inilparvovec; expectations regarding the adequacy of clinical data to support the marketing application and approval of ifezuntirgene inilparvovec; expectations regarding timing of receiving potential approval of, ifezuntirgene inilparvovec; whether the FDA will hold an advisory committee to discuss the BLA and the timing of such review and approval based on the Prescription Drug User Fee Act ("PDUFA") target action date of May 1, 2027 for the BLA; and its belief related to the potential for ifezuntirgene inilparvovec, if approved, to meaningfully reduce disease progression based on the Company's evaluation and view of certain measurements of disease progression. 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: the risk that the FDA ultimately concludes that the Phase I/II trial data and/or the confirmatory trial data are not sufficient to support accelerated approval; the risk that more patient data become available that results in a different interpretation than the one derived from the year three and four data analyses, respectively; risks related to the Company's interactions with regulatory authorities, which may affect the pathways to regulatory approval; 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; and the Company's ability to continue to build and maintain the infrastructure and personnel needed to commercialize ifezuntirgene inilparvovec, if approved. 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 Forms 10-Q filed with the SEC on May 5, 2026 and July 29, 2026, respectively, 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.