uniQure Announces Positive Recommendation from Data Safety Monitoring Board of Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

~ No Significant Safety Concerns Observed ~

~ Independent Data Safety Monitoring Board Recommends Proceeding with Study Enrollment ~

~ Full Enrollment of First Cohort Expected Mid-2021 ~

LEXINGTON, Mass. and AMSTERDAM, The Netherlands, Feb. 08, 2021 (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 independent Data Safety Monitoring Board (DSMB) overseeing the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease has met and reviewed the six-month safety data from the first two enrolled patients and the 90-day safety data from the next two enrolled patients in the study. No significant safety concerns were noted to prevent further dosing, and the final six patients in the first cohort are now cleared for enrollment. uniQure expects to achieve full patient enrollment in cohort one by mid-year 2021. The Phase I/II study is a double-blind, randomized clinical trial being conducted in the United States. To date, two patients have been treated with AMT-130, and two patients received the imitation surgery.

"We are encouraged with the positive recommendation to proceed with patient enrollment from this second DSMB meeting," said Ricardo Dolmetsch, president of research and development at uniQure. "We will now focus on enrolling the last six additional patients in the first cohort and look forward to sharing initial biomarker and imaging data towards the end of the year."

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

The Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease will explore the safety, tolerability, and efficacy signals in 26 total patients with early manifest Huntington's disease split into a 10 patient, low-dose cohort followed by a 16 patient, higher-

dose cohort randomized to treatment with AMT-130 or an imitation (sham) surgery. The five-year, multi-center trial consists of a blinded 12-month core study period followed by unblinded long-term follow-up. Patients will receive a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). Additional details are available on www.clinicaltrials.gov (NCT04120493).

AMT-130 is uniQure's first clinical program focusing on the central nervous system (CNS) incorporating its proprietary miQURE™ platform.

About Huntington's Disease
Huntington's disease is a rare, inherited neurodegenerative disorder that leads to motor symptoms including chorea, and 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. Despite the clear etiology of Huntington's disease, there are no currently 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. We are leveraging our modular and validated technology platform to rapidly advance a pipeline of proprietary gene therapies to treat patients with hemophilia B, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. 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. These forward-looking statements include, but are not limited to, whether we will be able to enroll the next six patients in the clinical trial by mid-year 2021 and whether we will be able to announce initial biomarker and imaging data by the end of 2021. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's periodic securities filings, including its Annual Report on Form 10-K filed March 2, 2020 and Quarterly Report on Form 10-Q filed on October 27, 2020. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

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

FOR MEDIA:

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