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

November 2, 2021

~ No Significant Safety Concerns Observed in First Four Patients Enrolled in Higher-dose Cohort ~

~ Enrollment Expected to be Completed by Mid-2022 ~

LEXINGTON, Mass. and AMSTERDAM, Nov. 02, 2021 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the positive recommendation by the independent Data Safety Monitoring Board (DSMB) following a review of safety data from the four patient procedures in the higher-dose cohort of the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease. With the positive recommendation, the final 12 patients in this second cohort are now cleared for enrollment.

A total of 14 blinded administration procedures have been completed as of August 2021. In the study to date, eight patients have been treated with AMT-130, and six patients have received imitation surgery. Full enrollment of the second, higher-dose cohort is expected to be completed in mid-2022.

"The AMT-130 program continues to advance at a steady pace, and with this positive recommendation from the DSMB, we are eager to enroll the final 12 patients in the higher-dose cohort," said David Cooper, M.D., vice president of clinical development at uniQure. "We also look forward to expanding our efforts with the initiation of a separate open-label study of AMT-130 in Europe and to sharing preliminary imaging and biomarker data from the initial four patients in the U.S. clinical trial before the end of the year."

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

The U.S. Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease is exploring the safety, tolerability, and efficacy signals in a planned 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; patients will be randomized to treatment with AMT-130 or an imitation (sham) surgery. The multi-center trial consists of a blinded 12-month core study period followed by unblinded long-term follow-up for five years. A total of 16 patients in the clinical trial 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).

The European, open-label Phase Ib/II study of AMT-130 will enroll 15 patients with early manifest Huntington's disease across two dose cohorts. Together with the U.S. study, the European study is intended to establish safety, proof of concept, and the optimal dose of AMT-130 to take forward into Phase III development or into a confirmatory study should an accelerated registration pathway be feasible.

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 commence the enrollment of the final 12 patients in the second dose cohort in the fourth quarter of 2021 or ever, whether we complete enrollment of the U.S. Phase I/II clinical trial of AMT-130 by the middle of 2022 or ever, whether we initiate dosing in our European open-label Phase Ib/II clinical trial in the second half of 2021 or ever, whether we are able to enroll the currently planned number of patients in the U.S. Phase I/II or the European open-label Phase Ib/II clinical trials, , and whether we share initial imaging and biomarker data towards the end of the year or ever. 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, manufacturing activities, 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 25, 2021. 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.

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