uniQure Announces Enrollment of Next Two Patients in Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

Lexington, MA and Amsterdam, the Netherlands, October 13, 2020 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that two additional patient procedures have been completed in the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease. The ongoing patient enrollment follows a meeting last month of the trial's independent Data Safety Monitoring Board (DSMB) to review 90-day follow-up data from the first two patients. The DSMB observed no significant safety concerns to prevent further dosing. The Phase I/II study is a double-blinded, randomized and controlled clinical trial being conducted in the United States. A total of four patients have been enrolled in the study thus far, including two patients treated with AMT-130 and two patients who received imitation surgery.

"We are very pleased with the progress being made to advance this first-in-human AAV gene therapy for Huntington's disease," said Ricardo Dolmetsch, Ph.D., president of research and development at uniQure. "This is an important achievement that puts us on our original clinical development timeline, making up for the modest delay in the study earlier this year due to COVID-19. In accordance with the study protocol, patient enrollment is expected to continue after a DSMB meeting to review 90-day follow-up data on these two new patients and 6-month data on the first two patients. We expect that this DSMB review will take place early next year and that patient enrollment in the 10-patient first dose cohort will be completed by mid-2021."

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 patients with early manifest Huntington's disease randomized to treatment with AMT-130 or an imitation (sham) surgery across two dose cohorts. The multi-center trial consists of a blinded 12-month core study period followed by unblinded long-term follow-up for 5 years after administration of AMT-130. 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 patient enrollment will continue after a DSMB meeting to review follow-up data, whether the DSMB review will take place early next year, and whether patient enrollment in the first dose cohort will be completed by mid-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 July 30, 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.

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