



uniQure Announces Completion of Enrollment in First Cohort of Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

~ Enrollment of second dose cohort expected to begin in 3Q 2021 ~

~ Company announces plans to initiate a second clinical study of AMT-130 in Europe in the second half of 2021 ~

Lexington, MA and Amsterdam, the Netherlands, April 5, 2021 — [uniQure](#) N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the completion of patient enrollment in the first dose cohort of a randomized, double-blinded, Phase I/II clinical trial of [AMT-130 for the treatment of early stage Huntington's disease](#). The Company also announced plans to begin an open-label clinical trial of AMT-130 in Europe later this year.

"This is an important milestone in our ongoing clinical development of AMT-130," stated David Cooper, M.D., vice president, clinical development at uniQure. "Nine U.S. study sites are now active to support enrollment in the next cohort, which is expected to start after the Data Safety Monitoring Board's review in the middle of the year. Completing enrollment of the first, 10-patient cohort ahead of schedule highlights the high level of interest among the Huntington's disease patient and clinical community, and the collaboration between our participating HD Centers of Excellence and the expert neurosurgical sites performing the MRI-guided procedures. We also look forward to initiating a new clinical study of AMT-130 in Europe later this year. It is estimated that there could be as many as 75,000 Europeans affected by Huntington's disease."

The ongoing [Phase I/II clinical trial of AMT-130](#) is a randomized, sham controlled, double-blinded study to explore the safety, tolerability, and proof of concept of AMT-130 in patients with early manifest Huntington's disease. The study, which includes two dose cohorts, will randomize a total of 26 patients to either treatment with AMT-130 or an imitation surgical procedure. The first dose cohort includes 10 patients, of which six patients received treatment with AMT-130 and four patients received imitation surgery. The second dose cohort is planned to include 16 patients, of which 10 patients will receive treatment with AMT-130 and six patients will receive imitation surgery. The trial consists of a blinded 12-month study period followed by unblinded long-term follow-up for 5 years after administration of AMT-130. Patients receive a single administration of AMT-130 through [MRI-guided, convection-enhanced stereotactic neurosurgical delivery](#) directly into the striatum (caudate and putamen).

The planned Phase Ib/II study of AMT-130 will be conducted in Europe and is expected to begin enrolling patients in the second half of 2021. This open-label study 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 comprises a recombinant AAV5 vector carrying a DNA cassette encoding a microRNA that lowers Huntingtin protein in Huntington's disease patients. AMT-130 is uniQure's first clinical program incorporating its proprietary miQURE™ platform. miQURE is designed to degrade disease-causing genes without off-target toxicity and induce silencing of the entire target organ through secondary exosome-mediated delivery.

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, the enrollment of patients in, or Data Safety Monitoring Board review of, our Phase I/II gene therapy clinical trial of AMT-130 in Huntington's disease, including whether we will be able to fully enroll the second dose cohort as currently planned, and whether we will initiate our P1b/II clinical study of AMT-130 in Europe later this year or ever. uniQure's 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 Commercialization and License Agreement with CSL Behring, the regulatory approval of that transaction, 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 1, 2021. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and uniQure assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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