



uniQure Announces Positive Recommendation to Advance Phase I/II Clinical Trial of AMT-130 for the Treatment of Huntington's Disease

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Independent Data Safety Monitoring Board Recommends Proceeding with Second, Higher-Dose Cohort

LEXINGTON, Mass. and AMSTERDAM, The Netherlands, May 27, 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 safety data for the fully enrolled first cohort of ten patients. This data set included nine-month safety data from the first two enrolled patients, six-month safety data from the next two enrolled patients, and 30-day safety data on remaining six patients in the trial. The DSMB recommended continued dosing in the study, and uniQure will now begin to enroll patients in the higher-dose cohort of the trial. The Phase I/II study is a double-blind, randomized clinical trial being conducted in the United States. To date, six patients have been treated with AMT-130, and four patients received the imitation surgery.

"We are very pleased with the progress of the study and with the recommendations of the DSMB. We look forward to proceeding with dose-escalation in the second cohort of patients," said [Ricardo Dolmetsch, Ph.D., president of research and development at uniQure](#). "We remain highly focused on progressing this therapy given the significant unmet need in the Huntington's disease community and are committed to initiating clinical development of AMT-130 in Europe in the second half of this year and to sharing initial imaging and biomarker data from 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 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 multi-center trial consists of a blinded 12-month core study period followed by unblinded long-term follow-up for five years. 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).

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 begin dosing the second cohort of the U.S. Phase I/II clinical study of AMT-130, whether we initiate dosing in our European open-label Phase Ib/II in the second half of 2021 or ever, 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 Commercialization and License Agreement with CSL Behring, 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 May 10, 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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