uniQure Receives FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for Investigational Gene Therapy AMT-130 in Huntington's Disease

June 3, 2024

~ Designation based on 24-month interim Phase I/II clinical data for AMT-130 announced in December 2023 ~

~ AMT-130 is the first therapeutic candidate to receive RMAT Designation for Huntington's disease ~

~ Receiving RMAT designation enables increased collaboration with the FDA on regulatory approval planning, in addition to the opportunity for expedited product development ~

LEXINGTON, Mass. and AMSTERDAM, June 03, 2024 (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 U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation for its investigational gene therapy AMT-130 for the treatment of Huntington's disease.

The FDA granted RMAT designation based on the potential of AMT-130 to address the major unmet medical need among patients with Huntington's disease. The designation follows the FDA's review of interim Phase I/II clinical data for AMT-130 announced in December 2023 and is based on an analysis comparing these 24-month clinical data to a non-concurrent criteria-matched natural history cohort.

"We're thrilled to receive the first ever RMAT designation for an investigational therapy for Huntington's disease," stated Matt Kapusta, chief executive officer of uniQure. "This achievement is a significant milestone for the program and supports the potential for AMT-130 to address the high unmet medical need of those suffering from this devastating disease."

"The RMAT designation, which was based on the comparison of the two-year AMT-130 data to a natural history cohort, marks a promising start to our FDA interactions," stated Walid Abi-Saab, chief medical officer of uniQure. "Importantly, RMAT designation allows for increased collaboration with the FDA to accelerate development, potentially facilitating earlier access for patients with life-threatening medical conditions. I'm incredibly proud of the team at uniQure for this accomplishment, and we look forward to presenting updated interim data from our ongoing Phase I/II studies in the middle of the year."

RMAT designation was created as part of the 21st Century Cures Act to expedite the development and review processes of regenerative medicine therapies. A regenerative medicine therapy can be eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious condition, and if preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a condition. Receiving RMAT designation offers sponsor companies all the benefits of the fast track and breakthrough therapy designation programs, allowing for early, close and frequent interactions with the FDA. This includes opportunities for early agency interactions to discuss the use of surrogate or intermediate endpoints, potential approval pathways including ways to support accelerated approval, and other opportunities to expedite development.

uniQure expects to provide updated interim data from the ongoing Phase I/II U.S. and European studies of AMT-130 in the middle of 2024. The interim update will include up to three years of follow-up on 29 treated patients, 21 of which will have been followed for at least two years.

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 26 patients with early manifest Huntington's disease split into a 10-patient low-dose cohort followed by a 16-patient high-dose cohort. Patients are 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 a period of up five years. A total of 16 patients in the clinical trial were randomized to treatment and received a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). An additional four control patients in the high-dose cohort crossed over to treatment following the core study period. Additional details are available on www.clinicaltrials.gov (NCT04120493).

The European, open-label Phase Ib/II study of AMT-130 enrolled 13 patients with early manifest Huntington's disease across two dose cohorts; a low-dose cohort of six patients and a high-dose cohort of seven patients. 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.

Patient dosing is ongoing in a third cohort of up to 12 patients to further evaluate both doses of AMT-130 in combination with perioperative immunosuppression. Enrollment in this third cohort is expected to be completed in the second half of 2024. AMT-130 is uniQure's first clinical program focusing on the central nervous system 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, 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 currently no approved therapies to delay the onset or to slow the disease's progression.

About uniQure

uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy for hemophilia B – a historic achievement based on more than a decade of research and clinical development – represent a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology and manufacturing platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (ALS), Fabry disease, and other severe 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," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "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. Examples of these forward-looking statements include, but are not limited to, statements concerning the potential of AMT-130 to address the unmet medical needs of Huntington's disease patients; the potential that RMAT designation will expedite the ongoing development of AMT-130 and facilitate earlier access to Huntington's disease patients; the Company's plans to present updated interim data from the ongoing Phase I/II U.S. and European studies of AMT-130 in mid-2024; and the Company's plans regarding the third cohort in its AMT-130 clinical trial and the timing of enrollment for such cohort. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, among others: risks associated with the Company's clinical results and the development and timing of its programs; the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to regulatory approval; whether RMAT designation or any accelerated pathway will lead to regulatory approval; the Company's ability to conduct and fund a Phase III or confirmatory study for AMT-130; the Company's ability to continue to build and maintain the company infrastructure and personnel needed to achieve its goals; the Company's effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of gene therapies; the Company's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; the Company's ability to obtain, maintain and protect its intellectual property; and the Company's ability to fund its operations and to raise additional capital as needed. These risks and uncertainties are more fully described under the heading "Risk Factors" in the Company's periodic filings with the U.S. Securities & Exchange Commission ("SEC"), including its Annual Report on Form 10-K filed February 28, 2024 and in other filings that the Company makes with the SEC from time to time. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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