uniQure Receives FDA Fast Track Designation for AMT-130 Gene Therapy for the Treatment of Huntington's Disease

~ On Track to Treat First Patient in Phase I/II Study of AMT-130 in 2H19 ~

Lexington, Mass. and Amsterdam, the Netherlands, April 8, 2019 — 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 Fast Track designation for AMT-130, the Company's gene therapy candidate for the treatment of Huntington's disease. AMT-130 comprises a recombinant AAV5 vector carrying a DNA cassette encoding a microRNA that non-selectively lowers or knocks-down human huntingtin protein in Huntington's disease patients. AMT-130 also is unique in that it targets the highly toxic exon1 protein fragment that is even more toxic than the mutant huntingtin protein.

"Achieving Fast Track Designation from the FDA underscores the high unmet medical need for patients suffering from Huntington's disease, for which there are currently no approved, disease-modifying treatments," stated Matt Kapusta, chief executive officer of uniQure. "We are nearing the initiation of a Phase I/II study of AMT-130, the first one-time administered AAV gene therapy to enter clinical testing for Huntington's disease, and are on track to treat the first patient in the second half of 2019."

The FDA's Fast Track program is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. A therapy granted Fast Track Designation may be eligible for several benefits, including more frequent meetings and communications with the FDA and, if relevant criteria are met, the potential for Accelerated Approval, Priority Review or Rolling Review of a Biologics License Application (BLA) or New Drug Application (NDA).

About Huntington's Disease

Huntington's disease is a rare, inherited neurodegenerative disorder that leads to loss of muscle coordination, behavioral abnormalities and cognitive decline, resulting in complete 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 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 and partnered gene therapies to treat patients with hemophilia, Huntington's disease and other severe genetic 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, our ability to initiate dosing of a Phase I/II study of AMT-130 in the second half of 2019 or ever, and our ability to open clinical sites for the Phase I/II study in the United States. Our actual results could differ materially from those anticipated in these forward-looking

statements for many reasons, including, without limitation, risks associated with our and our collaborators' clinical development activities, clinical results, collaboration arrangements, corporate reorganizations and strategic shifts, 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 Annual Report on Form 10-K filed on February 28, 2019. 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.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor
Direct: 339-970-7536
Mobile: 617-680-9452
m.cantor@uniQure.com

Eva M. Mulder
Direct: +31 20 240 6103
Mobile: +31 6 52 33 15 79
e.mulder@uniQure.com

FOR MEDIA:

Tom Malone
Direct: 339-970-7558
Mobile:339-223-8541
t.malone@uniQure.com