uniQure Announces Updated Clinical Data from Phase IIb Study of AMT-061 in Patients with Hemophilia B

~ Increases in FIX Activity Sustained at up to 51% of Normal, with Mean FIX of 38% of Normal at Twelve Weeks After Administration ~

~ None of the Patients Received Factor Infusions, Reported Bleeding Events or Required Immunosuppression Over a Combined 42 Weeks of Observation ~

~ Company Hosting Investor Conference Call and Webcast Today at 8:30 a.m. EST ~

Lexington, MA and Amsterdam, the Netherlands, February 8, 2019 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced updated clinical data in patients treated in the Company's ongoing Phase IIb study of AMT-061, an investigational AAV5-based gene therapy containing a patent-protected FIX-Padua variant, for the treatment of patients with severe and moderately severe hemophilia B. These data were presented in an oral session at the Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) and show that therapeutic levels of Factor IX (FIX) activity continue to be sustained in all three patients up to sixteen weeks after a single administration of AMT-061.* AMT-061 has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration and access to the Priority Medicine (PRIME) regulatory initiative by the European Medicines Agency.

The Phase IIb study of AMT-061 is an open-label, single-dose, single-arm, multi-center trial being conducted in the United States. Three patients with severe hemophilia (endogenous FIX activity less than one percent) were enrolled in the study and received a single intravenous infusion of $2x10^{13}$ vc/kg. Prior to the administration of AMT-061, all three patients showed low levels of pre-existing antibodies to AAV5 but were not excluded from the trial on that basis.

Updated data presented at EAHAD show that all three patients have demonstrated increasing and sustained FIX levels after the one-time administration of AMT-061. Mean FIX activity for the three patients at twelve weeks increased to 38% of normal, exceeding threshold FIX levels generally considered sufficient to eliminate or significantly reduce the risk of bleeding events. The first patient achieved FIX activity of 48% of normal at sixteen weeks after administration. FIX activity in the second patient was 25% of normal at fourteen weeks after administration, and the third patient achieved FIX activity of 51% of normal at twelve weeks after administration. The second and third patients had previously screen-failed and were excluded from another gene therapy study due to pre-existing neutralizing antibodies to a different AAV vector. Reported FIX activity was measured using an activated partial thromboplastin time (aPTT) assay performed at a central laboratory.

"We are extremely pleased with these updated data," stated Robert Gut, M.D., Ph.D., chief medical officer of uniQure. "The study demonstrates AMT-061 has the potential to increase FIX activity into the normal range and continues to be very well tolerated, with no serious adverse events reported and no patients requiring any immunosuppression therapy. We look forward to providing further updates on these patients later in the year at other academic conferences."

*** Epidemiological data indicate that factor activity above 12% of normal is associated with substantial reduction or elimination of spontaneous bleeds and factor usage. Den Uijl IE et al Haemophilia 2011; 17(6):849-53**

No patient in the study has experienced a material loss of FIX activity, reported any bleeding events or required any infusions of FIX replacement therapy. As previously reported, one patient experienced slight elevations in aspartate aminotransferase (AST), which quickly resolved without any additional treatment or loss of FIX activity. No patient has experienced any material elevation in alanine aminotransferase (ALT) after the administration of AMT-061.

"Our goal with AMT-061 is to give all people living with hemophilia B access to a one-time treatment capable of normalizing FIX activity and eliminating the need for replacement therapy, without the risk of immune responses that require immunosuppression or may lead to a loss of efficacy," stated Matt Kapusta, chief executive officer of uniQure. "These updated data continue to suggest that AMT-061 may be the first gene therapy able to achieve this goal, and we remain focused on completing enrollment in our ongoing pivotal Phase III study by the end of the year."

Patients in the Phase IIb study will be followed for 52 weeks to assess FIX activity, bleeding rates and usage of FIX replacement therapy, and will be monitored for five years to evaluate the safety of AMT-061.

Investor Conference Call and Webcast Information

uniQure will host a conference call today, February 8, 2019 at 8:30 a.m. ET, to discuss these updated clinical data from the Phase IIb study of AMT-061. To access the call by phone, dial 1-866-966-1396 (United States) or +44 (0) 20 719 280 00 (international); the conference ID is 3588567.

The webcast may also be accessed through the Investors section of the Company's website at www.uniQure.com. Following the live webcast, a replay of the call will be available for two weeks.

About AMT-061

AMT-061 consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). uniQure holds multiple issued patents in the United States and Canada broadly covering methods of treating bleeding disorders, including hemophilia B, using AAV gene therapy with the FIX-Padua variant. Additional patents are pending in the European Union.

AAV5-based gene therapies have been demonstrated to be safe and well-tolerated in a multitude of clinical trials, including four uniQure trials conducted in 25 patients in hemophilia B and other indications. No patient treated in clinical trials with the Company's AAV5 gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

About the Pivotal, Phase III HOPE-B Study

The pivotal, Phase III HOPE-B trial is a multinational, multi-center, open-label, single-arm study to evaluate the safety and efficacy of AMT-061. Approximately 50 adult hemophilia B patients classified as severe or moderately severe will be enrolled in a six-month observational period during which time they will continue to use their current standard of care to establish a baseline control. After the six-month lead-in period, patients

will receive a single intravenous administration of AMT-061. Dosing of patients in the HOPE-B pivotal trial is now underway.

The primary endpoint of the study will be based on the FIX activity level achieved following the administration of AMT-061, and the secondary endpoints will measure annualized FIX replacement therapy usage, annualized bleed rates and safety.

Patients enrolled in the HOPE-B pivotal trial will be tested for the presence of pre-existing neutralizing antibodies to AAV5 but will not be excluded from the trial based on their titers. Previous studies performed by uniQure suggest that AAV5 gene therapies may be viable treatments for at least 97% of patients.

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, the completion of our Phase IIb study, our ability to achieve the target profile for AMT-061, our ability to provide all people living with hemophilia B access to a one-time treatment capable of normalizing FIX activity and eliminating the need for replacement therapy, without the risk of immune responses that require immunosuppression or may lead to a loss of efficacy, the ability of AMT-061 to deliver functionally curative increases in FIX activity or to provide a favorable immunogenicity profile or to eliminate the risk of an immune response that may lead to a loss of efficacy or to expand patient eligibility for treatment with gene therapy or to be the first to market, the achievement of any of our planned near term or other milestones, our ability to provide further clinical updates on the Phase IIb study at medical conferences in 2019 or at any time, our ability to complete enrollment in our pivotal Phase III trial of AMT-061, the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies such as the dosing of patients in the HOPE-B pivotal trial in the first quarter of 2019 or at any time, and/or the development and regulatory approval of our product candidates in the United States or in Europe. 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 Quarterly Report on Form 10-Q filed on November 6, 2018. 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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