uniQure Announces Initial Topline Data from Dose-Confirmation Study of AMT-061 in Patients with Hemophilia B

~ All Patients Achieved and Sustained Therapeutic Factor IX Activity with a Mean FIX Level of 31% of Normal at Six Weeks After Administration ~

~ None of the Patients Received Factor Infusions, Experienced Any Reported Bleeding Events or Required Immunosuppression Over a Combined 24 Weeks of Observation ~

~ Company R&D Day Scheduled for Monday, November 19 at 8:30 a.m. EST ~

Lexington, MA and Amsterdam, the Netherlands, November 15, 2018 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced initial clinical data in patients treated in the Company's Phase IIb dose-confirmation 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 show that therapeutic levels of Factor IX (FIX) activity have been achieved and sustained in all three patients at six weeks after a single administration of AMT-061.* AMT-061 has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration (FDA) and access to the Priority Medicine (PRIME) regulatory initiative by the European Medicines Agency (EMA).

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 were enrolled in this study and received a single intravenous infusion of $2x10^{13}$ vc/kg. Patients are evaluated for the presence of pre-existing neutralizing antibodies to AAV5 but not excluded from the trial on that basis. The objective of the study is to evaluate the safety and tolerability of AMT-061 and confirm the dose based on FIX activity at six weeks after administration. Patients in the 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.

Data from the study show that all three patients experienced increasing and sustained FIX levels after a one-time administration of AMT-061. Six weeks after administration mean FIX activity for the three patients was 31% of normal, exceeding threshold FIX levels generally considered sufficient to significantly reduce the risk of bleeding events. FIX activity was measured using an activated partial thromboplastin time (aPTT) assay performed at a central laboratory. The first patient achieved FIX activity of 37% of normal at ten weeks after administration. FIX activity in the second patient was 23% of normal at eight weeks following administration and in the third patient was 30% of normal at six weeks after administration. The second and third patients had previously screen-failed another gene therapy study due to pre-existing neutralizing antibodies to a different AAV vector.

"We are highly encouraged by these early data, where all patients achieved therapeutic levels of FIX activity," stated Robert Gut, M.D., Ph.D., chief medical officer of uniQure. "Based on the long-term FIX data from our Phase I/II trial of AMT-060, where we saw FIX activity levels continue to increase beyond the levels achieved at six to ten weeks, we are hopeful that we will continue to see similar trends in these patients."

* 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

Based on the data obtained to date, no patient has experienced a material loss of FIX activity, reported any bleeding events or required any infusions of FIX replacement therapy. AMT-061 has been well-tolerated, with no serious adverse events reported and no patients requiring any immunosuppression therapy. One patient experienced a mild, asymptomatic and transient increase in liver enzyme levels, which resolved quickly without any additional treatment.

"The transition to our patent-protected Padua construct with AAV5 was undertaken with the goal of providing all hemophilia B patients an opportunity to access a one-time treatment capable of increasing FIX activity to potentially functionally curative levels without the risk of immune responses that may lead to a loss of efficacy," stated Matt Kapusta, chief executive officer of uniQure. "The initial data from our Phase IIb study suggest this target profile is achievable, and we look forward to providing further clinical updates on the study at medical conferences in 2019."

uniQure expects to submit data from the Phase IIb study to the FDA and EMA by the end of the year. The study's Data Monitoring Committee will meet before the end of the year to evaluate and potentially confirm the dose for the Phase III study. The Company expects to initiate the dosing phase of its Phase III HOPE-B pivotal study in the first quarter of 2019.

uniQure will host a Research & Development Day on Monday, November 19, 2018 in New York City, from 8:30 a.m. to 12:15 p.m. EST. The event will include presentations on new research programs that expand the Company's gene therapy pipeline, feature advancements in research technology and provide updates on current programs, including AMT-061.

About AMT-061

AMT-061 consists of an AAV5 vector carrying a gene cassette with the Padua variant of Factor IX (FIX-Padua). FIX-Padua has been reported to provide a gain of function compared to the wild-type FIX protein, which was used in AMT-060.

uniQure holds multiple issued patents in the United States, the European Union 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 United States and 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.

About the Phase III HOPE-B Pivotal Study

The Phase III HOPE-B pivotal 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 expected to start in the first quarter of 2019.

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 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 cardiovascular 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, the confirmation of the dose for AMT-061 and the timing of such confirmation, our plan to submit data to the FDA and EMA by the end of the year, our ability to achieve the target profile for AMT-061, the ability of AMT-061 to be a leading gene therapy treatment for all or nearly all hemophilia B patients or 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, the realization of FIX activity levels that continue to increase beyond the levels achieved at six to ten weeks or that follow previously observed patterns of activity, 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, 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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