uniQure Announces Long-Term Clinical Data from Ongoing Phase I/II Trial of AMT-060 and Confirms Dose for AMT-061 Pivotal Study in Hemophilia B

-- Clinical Benefit of AMT-060 Maintained in All Patients Through up to Two and a Half Years of Follow-up –

-- Second Dose Cohort Demonstrated a 93% Reduction in FIX Replacement Therapy Usage During Last Twelve Months of Observation, with Annualized Bleeds Near Zero --

-- Dose Confirmed for Phase III Pivotal Study of AMT-061, with Patient Dosing Expected to Commence in First Quarter of 2019 --

Lexington, MA and Amsterdam, the Netherlands, December 3, 2018 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced updated results from its ongoing Phase I/II trial of AMT-060, and provided an update on AMT-061, the Company's next-generation gene therapy candidate currently in late-stage clinical development for patients with hemophilia B. AMT-060 is a first-generation gene therapy consisting of an AAV5 vector carrying a gene cassette with the wild-type FIX gene. The data on AMT-060 includes up to two and a half years of follow-up from the low-dose cohort and up to two years of follow-up from the second, higher-dose cohort. These clinical data were presented on Sunday, December 2 in a poster presentation at the 59th American Society of Hematology (ASH) Annual Meeting taking place in San Diego, California.

AMT-060 continues to be safe and well-tolerated, with no new serious adverse events and no development of inhibitors. All 10 patients sustained increases in FIX activity and improvements in their disease state as measured by reduced usage of FIX replacement therapy and decreased bleeding frequency.

All five patients in the second dose cohort of $2x10^{13}$ gc/kg continue to be free of routine prophylaxis at up to two years after treatment. During the last 12 months of observation, the mean annualized bleeding rate was 0.5 bleeds, representing an 88% reduction compared to the year prior to treatment. During the same period, the usage of FIX replacement therapy declined 93% compared to the year prior to treatment. Mean FIX activity increased from 7.1% in the first year after treatment to 8.3% in the second year and was 8.9% of normal at the last measurement.

"With up to two and a half years of follow-up, patients in the study continue to show evidence of durable clinical benefits, including sustained FIX activity, substantial reductions in the usage of replacement therapy and a near cessation of spontaneous bleeds," stated Professor Frank W.G. Leebeek, M.D. Ph.D. of the Erasmus University Medical Center in Rotterdam, the Netherlands. "Most importantly, since the last data update at ASH 2017, the study continues to demonstrate the long-term safety and tolerability of AAV5-based gene therapies, with no new treatment-related adverse events, no toxic cellular immune responses and no patients losing FIX activity."

Advancing AMT-061 in Late-Stage Clinical Development

The Company recently announced initial clinical data in patients treated in a Phase IIb dose-confirmation study of AMT-061, a next-generation, AAV5-based gene therapy containing a patent protected FIX-Padua variant for the treatment of hemophilia B. Six weeks after administration, mean FIX activity for the three patients in the study was 31% of normal, and FIX levels continue to increase beyond the initial six to ten weeks of follow up. Based on the data obtained to date, no patient has required any infusions of FIX replacement therapy, reported any bleeding events or required any immunosuppression therapy.

The Data Monitoring Committee for the study has now reviewed these initial data and confirmed the dose of $2x10^{13}$ gc/kg for administration in the HOPE-B Phase III pivotal trial. The Company expects the dosing phase of the pivotal study to begin in the first quarter of 2019.

"The long-term data from our Phase I/II study of AMT-060 demonstrate the durability and safety of AAV5-based gene therapies and bode well for the late-stage development of AMT-061," stated Matt Kapusta, chief executive officer of uniQure. "We believe AMT-061, which has clinically demonstrated the potential to provide functionally curative increases in FIX activity, may be a best-in-class gene therapy for patients with hemophilia B, and we look forward to treating our first patient in the pivotal study early next year."

About AMT-060 and AMT-061

AMT-060 is a first-generation gene therapy consisting of an AAV5 vector carrying a gene cassette with the wild-type FIX gene. In October 2017, the Company announced the transition to AMT-061, which also consists of an AAV5 vector including the Padua variant of Factor IX (FIX-Padua). AMT-060 and AMT-061 are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. FIX-Padua has been reported in multiple preclinical and nonclinical studies to provide an approximately 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein.

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 reported cytotoxic T-cell-mediated immune response to the capsid.

About the Phase I/II study of AMT-060

The Phase I/II study is an open-label, multi-center study including 10 patients each receiving a one-time, 30-minute, intravenous administration of AMT-060, without the prophylactic use of corticosteroids. The study includes two dose cohorts of five patients each, with the first cohort receiving 5x1012 gc/kg and the second cohort receiving 2x1013 gc/kg. Nine patients in the trial were classified as having severe (<1% FIX activity) hemophilia. One patient in the low-dose cohort had a moderate/severe (1.5% FIX activity) phenotype.

About the Phase IIb study of AMT-061

The Phase IIb study 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 AMT-061 at a dose 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.

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 evaluated for the presence of pre-existing neutralizing antibodies to AAV5 but will not be excluded from the trial on that basis. Previous preclinical 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 B, hemophilia A, Huntington's disease, Fabry disease, Spinocerebellar ataxia Type 3, 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 ability of AMT-061 to be a successful gene therapy treatment for at least 97% hemophilia B patients or to deliver sustained increases in FIX activity or to provide a favorable immunogenicity profile or to eliminate the risk of an immune response, the realization of FIX activity levels that continue to be sustained or increased beyond the levels achieved in the AMT-060 and AMT-061 studies, the determination that any of AMT-060, AMT-061 and the AAV5 capsid used in those product candidates are safe or effective or will be determined by any regulatory body to be safe and effective, 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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