uniQure Announces First Clinical Data From Second Dose Cohort of AMT-060 in Ongoing Phase I/II trial in Patients with Severe Hemophilia B

-- Second-dose Cohort Demonstrates Dose Response with All Patients Free of Prophylactic FIX Replacement Therapy and Only One Spontaneous Bleed Reported

-- Low-Dose Cohort Shows Sustained Levels of FIX Activity Up To One Year, with Marked Reduction of Bleeding Over Time and a Complete Cessation of Spontaneous Bleedings in Last 14 Weeks of Observation

--No Activation of T-Cell Responses or Loss of FIX Activity in Any Patients

--Investor Webcast Monday, December 5, 2016 at 7:00 a.m. PST/10 a.m. EST

Lexington, MA and Amsterdam, the Netherlands, December 4, 2016 — uniQure N.V. (Nasdaq: QURE), a leader in human gene therapy, today announced new and updated results from its ongoing, dose-ranging Phase I/II trial of AMT-060, its proprietary, investigational gene therapy in patients with severe hemophilia B. The data includes up to 52 weeks of follow-up from the low-dose cohort and up to 31 weeks of follow-up from the second dose cohort.

New data presented from the second-dose cohort show a dose response with substantial improvement in disease state in all five patients, including the discontinuation of precautionary Factor IX (FIX) infusions in all four patients that previously required chronic replacement therapy. To date, only one spontaneous bleed was reported after discontinuation of prophylactic FIX replacement therapy.

All five patients in the low-dose cohort, who bleedings were previously uncontrolled despite being managed with prophylactic therapy, continue to maintain robust, constant and clinically meaningful levels of FIX activity for up to 52 weeks post treatment, with a complete cessation of spontaneous bleedings in the last 14 weeks of observation.

These clinical data from both patient cohorts were presented last evening in a poster session of the 58th American Society of Hematology (ASH) Annual Meeting taking place in San Diego, California.

"The data from this ongoing study demonstrate clinically significant and sustained increases in FIX activity, substantial reductions in FIX replacement usage and a near cessation of spontaneous bleeding episodes," stated Professor Frank W.G. Leebeek, M.D. Ph.D. of the Erasmus University Medical Center in Rotterdam, the Netherlands. "Importantly, at both doses evaluated, AMT-060 appears to be safe and well-tolerated with no loss of FIX activity, no activation of T-cell response and no development of inhibitors for any of the 10 patients in the study.

"In total, we are observing a therapeutic benefit from AMT-060 that is clearly superior to their previous prophylactic FIX replacement therapy regimen, even in patients with advanced joint disease who still experienced many bleeds despite prophylaxis with FIX," he added. "The safety and clinical efficacy profile observed in this study, together with the higher FIX expression levels support selection of the $2x10^{13}$ gc/kg dose for a pivotal registration trial."

Phase 1/2 Trial Overview

The AMT-060 gene therapy consists of a codon-optimized wild type FIX gene cassette, the LP1 liver promoter and an AAV5 viral vector manufactured by uniQure using its proprietary insect cell-based technology platform. It is the only hemophilia gene therapy that combines a gene cassette with clinically proven multi-year durability, now out more than five years, and an AAV5 vector serotype that has demonstrated safety and broad applicability due to the low prevalence of neutralizing antibodies (NABs) as evaluated in more than 20 patients across clinical studies in three different diseases.

- The Phase I/II, open-label, multi-center study includes 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 $5x10^{12}$ gc/kg and the second cohort receiving $2x10^{13}$ 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.

- Patients in the low-dose cohort were characterized by poorly controlled bleeding manifestations despite use of high-dose FIX replacement therapy during the year prior to study compared to the second-dose cohort.

- All but one patient in the study across both cohorts required chronic infusions of prophylactic FIX therapy at the time of enrollment. The remaining patient, who is in the

second dose cohort, used FIX therapy on demand.

- There were no screening failures due to pre-existing anti-AAV5 NABs in the study.

Key Data Update from Phase I/II Clinical Trial of AMT-060 in Hemophilia B Patients

Data as of October 15, 2016:

- All 10 patients in the study have demonstrated improvements in their disease state as measured by reduced FIX replacement therapy and bleeding frequency.

- In the second-dose cohort, only one spontaneous bleeding episode was reported over a period of 96 weeks of combined patient observation, representing a reduction in the annualized spontaneous bleed rate of 76% compared to the one-year period prior to administration of AMT-060.

- Bleeding data was also evaluated from the low-dose cohort. The frequency of spontaneous bleeds declined significantly over time, with no spontaneous bleeds reported by any patient in their last 14 weeks of observation. With up to 52 weeks of follow-up, the annualized spontaneous bleed rate for the four patients that discontinued prophylactic FIX infusions was reduced by 59% compared to the one year period prior to administration of AMT-060. The one patient in the low-dose cohort that remained on prophylaxis also experienced a 45% reduction in spontaneous bleeds.

- Eight of nine patients in the study that required chronic FIX infusions prior to administration of AMT-060, including all such patients in the second dose cohort, discontinued prophylaxis and remained prophylaxis-free at the last follow up (22-52 weeks).

- Among the four patients in the low-dose cohort that discontinued prophylaxis, annualized consumption of FIX concentrate following AMT-060 administration was reduced substantially by more than a cumulative total of 1,329,000 international units (85%), compared to their pre-trial usage levels. The one patient who remained on prophylactic FIX therapy in the low-dose cohort has reduced frequency of bleeding episodes and also requires materially less FIX concentrate after treatment with AMT-060.

- Through up to 6 months of follow-up among the five patients in the second-dose cohort, the mean steady-state FIX activity was approximately 7% of normal, with expression up to a FIX activity of 13% of normal.

- In both dose cohorts, FIX activity remained consistent and stable through up to one year of follow up with no emergence of late immune response or loss of FIX activity in any of the patients.

- AMT-060 continues to be well-tolerated, and there have been no severe adverse events.

- Three out of the total of 10 patients (two in the second-dose cohort and one previously reported from the low-dose cohort) experienced mild, asymptomatic elevations of alanine aminotransferase (ALT) and received a tapering course of corticosteroids per protocol. Importantly, the temporary elevations in ALT were not associated with any loss of endogenous FIX activity or T-cell response.

- No patients across either cohort have developed inhibitory antibodies against FIX, or demonstrated sustained AAV5 capsid-specific T-cell activation.

- AMT-060 continues to demonstrate a very low screening failure rate, with all patients screened in the study testing negative for pre-existing anti-AAV5 NABs. To date, 25 patients have been screened for pre-existing anti-AAV5 NABs with a fully validated assay across several clinical studies with only one patient excluded due to a borderline positive result. This collective data set suggests that a large proportion of the hemophilia patient population may be eligible for treatment with AMT-060.

"The data from our Phase I/II study demonstrate AMT-060 is delivering sustained and significantly improved clinical benefits to patients suffering from severe hemophilia B by enabling them to discontinue frequent infusions of FIX replacement therapy and to essentially eliminate the risk of spontaneous bleeding," stated Christian Meyer, M.D., Ph.D., chief medical officer at uniQure. "Importantly, none of the patients treated with AMT-060 have lost FIX activity for up to one year post administration. To date, our insect-cell manufactured AAV5 gene therapies have been administered to 22 patients across three clinical studies without any evidence of AAV5 capsid-specific cellular immune responses or long-term safety complications."

"The proprietary elements of AMT-060, including our fully-humanized FIX gene cassette and AAV5 vector, are the only gene therapy components clinically demonstrated in hemophilia B to be safe, effective, and durable for up to six and a half years," he added. "We believe these factors, along with our commercial-scale manufacturing capabilities, differentiate AMT-060 from other hemophilia gene therapies in development, and we look forward to advancing our program into a late-stage clinical study."

AMT-060 is being co-developed with Chiesi for Europe.

Investor/Analyst Breakfast and Webcast on Monday, December 5, 2016

uniQure management will host an investor breakfast meeting featuring Professor Leebeek to review the updated data on AMT-060 and outline progress taking place with its ongoing clinical development. The meeting will be webcast live along with slides and can be accessed by visiting the investor relations section of the Company's website at www.uniQure.com

Date and Time: Monday, December 5, 2016 at 7:00 am PST/ 10:00 am EST
Location: Marriott Gaslamp Hotel, Room Presidio C, 660 K Street, San Diego, CA 92101

To request attendance at the meeting, please RSVP to Investors@uniQure.com as space is limited

About Hemophilia B

Hemophilia B is a serious and rare inherited disease in males characterized by insufficient blood clotting. The condition can lead to repeated and sometimes life-threatening episodes of external and internal bleeding following accidental trauma or medical interventions. Severe hemophilia is characterized by recurrent episodes of spontaneous joint bleeds, that cause long-term damage to the joints resulting in disabling arthropathy. Bleeds may be fatal if they occur in the brain. The deficient blood clotting results from the lack of functional human Factor IX, or hFIX. Treatment of hemophilia B today consists of prophylactic or on-demand protein replacement therapy, in which one to three times weekly intravenous administrations of plasma-derived or recombinant hFIX are required to prevent bleeding and once daily infusions in case bleeding occurs. Hemophilia B occurs in approximately 1 out of 30,000 live births.

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 CNS, liver/metabolic and cardiovascular diseases. www.uniQure.com

uniQure Forward-Looking Statement

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, statements regarding the development of our gene therapy product candidates, including the future development of AMT-060, the success of our collaborations and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. 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, 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 2015 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 4, 2016. 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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