uniQure Announces Presentations at the 59th American Society of Hematology (ASH) Annual Meeting

~ New Nonclinical Data on AMT-061 to be Presented Saturday, December 9, 2017 ~

Lexington, MA and Amsterdam, the Netherlands, November 1, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced its company-sponsored clinical data presentations at the 59th American Society of Hematology (ASH) Annual Meeting, taking place in Atlanta, GA from December 9 to 12, 2017.

Poster Presentation

Title: *Predictable Protein Expression with Enhanced Factor IX Activity Following Administration of a Modified AAV5-hFIX Vector to Nonhuman Primates*
Presenter: Ying Poi Liu, Ph.D., senior scientist at uniQure
Session Name: 801. Gene Therapy and Transfer: Poster I
Date: Saturday, December 9, 2017
Presentation Time: 5:30 PM - 7:30 PM EST
Location: Georgia World Congress Center, Building A, Level 1, Hall A2

The conference abstract was made available today: ASH abstract.

A Good Laboratory Practices (GLP), nonclinical study of AMT-061 has been performed in non-human primates at four different dose levels up to a dose of 9×10^{13} gc/kg. The purpose of this study was to compare AMT-061 to AMT-060 with respect to liver transduction, circulating FIX protein levels, circulating FIX activity levels and toxicity, after a single intravenous dose with 13- or 26-week observation periods.

Data from the study demonstrated a strong correlation between dose and human FIX (hFIX) expression levels, as well as biological activity of the expressed hFIX protein. At equal doses, circulating vector DNA plasma levels, liver distribution, liver cell transduction and hFIX protein expression were comparable for both AMT-060 and AMT-061. Additionally, AMT-061 demonstrated substantial increases in FIX clotting activity compared to AMT-060, consistent with those previously reported for FIX-Padua.

Oral Presentation

Title: *Stable Elevations in FIX Activity and Reductions in Annualized Bleeding Rate over up to 2 Years of Follow-up of Adults with Severe or Moderate-Severe Hemophilia B Treated with AMT-060 (AAV5-hFIX) Gene Therapy*
Presenter: Professor Frank W.G. Leebeek, M.D. Ph.D.
Session Name: 801. Gene Therapy and Transfer: Gene Therapy for Hemophilia and Improving Lentiviral Vectors
Session Date: Monday, December 11, 2017
Presentation Time: 7:15 AM
Location: Georgia World Congress Center, Building C, Level 1, C101 Auditorium

The conference abstract was made available today: ASH abstract.

Long-term clinical data from the ongoing Phase I/II trial of AMT-060 in patients with severe hemophilia B will be presented on up to twenty-four months of follow-up. All ten patients in the study demonstrated improvements in their disease state as measured by reduced FIX replacement therapy and bleeding frequency. Across the clinical trials' two dosing cohorts, cumulative annualized FIX consumption decreased by 79% as of deadline for ASH abstract submission, and in the higher-dose cohort of the study, no spontaneous bleeds were reported in the last six months of follow-up - with a reduction in the annualized spontaneous bleed rate of 84% compared to the one-year period prior to gene transfer.

No patients developed inhibitors to FIX and there were no detectable signs of sustained AAV5 capsid-specific T-cell activation. Mild, temporary elevations in ALT were observed in three patients, none of which were associated with changes in in FIX activity or could be referred to as capsid-specific T-cell responses. ALT elevations have not recurred.

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. The episodes can cause long-term damage, for example to the joints, and can 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 frequent intravenous administrations of plasma-derived or recombinant hFIX are required to stop or prevent bleeding. 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 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 development of our gene therapy product candidates, 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, and the scope of protection provided by our patent portfolio. 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 Quarterly Report on Form 10-Q filed on November 1, 2017. 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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