# uniQure Announces Presentation of Non-Clinical Data of AMT-061 at the 59th American Society of Hematology (ASH) Annual Meeting

December 9, 2017

Non-human primate study of AMT-061 utilizing FIX-Padua shows 6.5-fold increase in FIX clotting activity compared to AMT-060

Data supports nonclinical comparability plan agreed upon with FDA

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Dec. 09, 2017 (GLOBE NEWSWIRE) -- uniQure N.V.(NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced new nonclinical data on AMT-061, its investigational gene therapy for patients with hemophilia B. AMT-061 combines an AAV5 vector with the FIX-Padua variant. The data will be presented today in a poster session at the 59th American Society of Hematology (ASH) Annual Meeting taking place in Atlanta, GA.

The data presented from the study in non-human primates demonstrated dose-dependent expression of human Factor IX protein (hFIX) and FIX clotting activity. Animals receiving the same dose of AMT-060 and AMT-061 showed comparable levels of hFIX protein, but the animals receiving AMT-061 demonstrated approximately 6.5-fold higher FIX activity compared to those receiving AMT-060.

"These data establish that AMT-061 is well-tolerated and provides a substantial increase in FIX activity compared to AMT-060, consistent with the gain-of-function reported in previous preclinical studies of FIX-Padua," stated Sander van Deventer, M.D., Ph.D., chief scientific officer of uniQure. "At a dose of 2x1013 gc/kg, the same dose used in the second patient cohort of our ongoing Phase I/II clinical trial of AMT-060, AMT-061 has the potential to achieve mean FIX activity in humans of approximately 30 to 50 percent of normal."

Key Data Findings Presented at ASH

The goal of this non-human primate study was to evaluate the efficacy and safety of introducing the FIX-Padua variant into the AMT-060 cassette. AMT-061 leverages the same AAV5 capsid and insect cell-based manufacturing platform as AMT-060. The study also compared AMT-061 to AMT-060 with respect to liver transduction, circulating FIX protein levels, circulating FIX activity levels and toxicity.

- Cynomolgus monkeys in 6 groups (*n* = 3 per group) received a one-time intravenous administration of either AMT-060 (5x1012 gc/kg), AMT-061 in a range of four doses (5x1011 to 9x1013 gc/kg) or the vehicle control.
- Animals receiving AMT-061 showed clear, dose-dependent increases in FIX protein and an approximately 6.5-fold increase in FIX clotting activity compared to AMT-060.
- At a dose of 5x1012 gc/kg, AMT-061 FIX activity was at average of 58.9% of normal in week 4 to week 13 post administration, whereas AMT-060 FIX activity was 9.1% of normal during this same time period. AMT-060 and AMT-061 showed similar circulating vector DNA plasma levels, liver distribution, liver cell transduction and transgene expression.
- Safety measures, including various markers of coagulation and fibrinolytic activation did not significantly differ between the groups, suggesting that there is no increased risk of thrombosis in animals expressing the Padua-FIX variant.
- No toxicological findings or target organ defects were detected after either AMT-060 or AMT-061 administration.

"The data confirms that the biological activity of AMT-060 and AMT-061 are highly comparable, but the FIX clotting activity of AMT-061 is substantially higher than AMT-060," added van Deventer. "We believe the enhanced potency of AMT-061 has the potential to provide substantial benefit to patients, and we look forward to advancing this promising gene therapy candidate into a pivotal study in 2018."

**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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