uniQure Announces Publication in the Journal Blood of Clinical Data from Phase I/II Trial of AMT-060 In Patients with Severe Hemophilia B

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Data Demonstrate Clinical Effectiveness and Superior Immunogenicity Profile of AAV5 Gene Therapy in Severe and Moderate-Severe Hemophilia B Patients

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Dec. 18, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the online publication in *Blood* of clinical data from the ongoing Phase I/II trial of AMT-060 in patients with severe hemophilia B. The manuscript reports data on up to one year of follow-up from the low-dose cohort of patients in the trial and up to six months of follow-up on patients in the higher-dose cohort.

The published manuscript, entitled "Gene therapy with adeno-associated virus vector 5-human factor IX in adults with hemophilia B," shows that AAV5 liver-directed wildtype hFIX gene transfer was well tolerated and clinically effective in patients with severe and moderate-severe hemophilia B. No cellular immune responses to the AAV5 vector were detected and Factor IX (FIX) expression levels were stable over the entire observation period. The manuscript is available online today and will be included in a future print edition of *Blood.*

The study included ten adults with hemophilia B and severe-bleeding phenotype. A single infusion of AMT-060 had a favorable safety profile and resulted in stable and clinically-important FIX activity increases, along with a marked reduction in spontaneous bleeds and FIX concentrate use, without detectable cellular-immune responses against capsids. In the higher-dose cohort, mean FIX activity increased to 6.9% of normal. Annualized FIX use decreased by 73%, and mean annual bleeding rate declined by 70%, from 3.0 to 0.9. FIX activity was stable in both cohorts and eight of nine participants receiving FIX at study entry stopped prophylaxis.

"*Blood*'s publication of this manuscript is a testament to the quality of the study and its significance to the advancement of gene therapy in hemophilia," stated Professor Wolfgang Miesbach, M.D., of the University Hospital Frankfurt, Germany. "We're very pleased to have these data published in a journal as highly regarded as *Blood*."

Additional follow-up on the patients from this study was presented on December 11, 2017, at the American Society of Hematology (ASH) Annual Meeting. The AAV5-based AMT-060 remains safe and well-tolerated with now up to two years of follow-up in the low-dose cohort and 1.5 years in the higher-dose cohort. There were no new serious adverse events and no development of inhibitors. To date, no patient in the study has had any loss of Factor IX activity or encountered a capsid-specific, T-cell-mediated immune response.

uniQure is preparing to initiate a pivotal study in 2018 with AMT-061, which combines an AAV5 vector with the FIX-Padua mutant. AMT-061 and AMT-060 are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. The gene variant, referred to as FIX-Padua, has been reported in multiple preclinical and nonclinical studies to provide an approximate 8 to 9-fold increase in FIX clotting activity compared to the wild-type FIX gene. All other critical quality attributes of AMT-061 are expected to be comparable to those of AMT-060, as AMT-061 utilizes the same AAV5 capsid and proprietary insect cell-based manufacturing platform.

"The data from the Phase I/II trial show that our AAV5-based gene therapies offer multi-year durability and a favorable immunogenicity profile, enabling hemophilia B patients to discontinue frequent infusions of FIX replacement therapy and to reduce the risk of spontaneous bleeding," stated Dr. Steven Zelenkofske, chief medical officer of uniQure. "With AAV5 emerging as a potential best-in-class vector for systemic administration to the liver, we look forward to advancing AAV5-FIX-Padua (AMT-061) into a pivotal trial in 2018."

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 transition to our AMT-061 product candidate, 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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