



uniQure Enrolls First Patient in Phase III HOPE-B Pivotal Study of AMT-061 in Patients with Hemophilia B

Lexington, MA and Amsterdam, the Netherlands, June 28, 2018 — [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced that it has enrolled its first patient in the Phase III HOPE-B pivotal study of AMT-061, an investigational AAV5-based gene therapy incorporating the FIX-Padua variant for the treatment of patients with severe and moderately severe hemophilia B.

AMT-061 has been granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration and access to the Priority Medicines (PRIME) regulatory initiative by the European Medicines Agency.

“AMT-061 has the potential to be a major advancement in gene therapy for patients affected by hemophilia B,” stated Steven Pipe, M.D., professor of pediatrics and pathology and pediatric medical director of the hemophilia and coagulation disorders program at the University of Michigan and principal investigator of the HOPE-B clinical trial. “A one-time treatment, such as AMT-061, could be life-changing for these patients, many of whom struggle to manage ongoing challenges, including compliance with frequent infusions and recurrent episodes of bleeding.”

“We are delighted to have enrolled the first patient in this Phase III pivotal study of a gene therapy for patients with hemophilia B,” said Steve Zelenkofske, D.O., chief medical officer at uniQure. “This represents a significant milestone for uniQure as we advance a potentially best-in-class gene therapy for patients with this life-altering disorder. In addition to advancing our pivotal trial, we have also initiated patient recruitment for our Phase IIb dose-confirmation study and expect to commence enrollment in July. We look forward to announcing top-line FIX data from the dose-confirmation study before the end of the year.”

HOPE-B Study Design

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 early in the first quarter of 2019.

The primary endpoint of the study will be based on the Factor IX (FIX) activity level achieved following the administration of AMT-061, and the secondary endpoints will measure annualized FIX replacement therapy use rate and annualized bleed rate.

Patients enrolled in the HOPE-B trial will be tested for the presence of pre-existing neutralizing antibodies to AAV5 but will not be excluded from the trial based on their titers. Previous studies performed by uniQure suggest that AAV5 gene therapies may be viable treatments for at least 97% of patients.

Concurrent with the lead-in phase of the HOPE-B pivotal study, uniQure will also conduct a short, Phase IIb dose-confirmation study of AMT-061 in approximately three patients. Patients will receive a single dose of 2×10^{13} vc/kg and be evaluated for a period of approximately six to eight weeks to determine FIX activity and confirm the dose of AMT-061 for the pivotal study. Patients enrolled in this dose-confirmation study will be followed for one year.

About AMT-061

AMT-061 consists of an AAV5 viral vector carrying a gene cassette with the Padua variant of Factor IX (FIX-Padua). FIX-Padua has been reported to provide an approximate 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein, as used in AMT-060. AAV5-based gene therapies have been demonstrated to be safe and well-tolerated in a multitude of clinical trials, including three uniQure trials conducted in 22 patients in hemophilia B and other indications. No patient treated in clinical trials with the Company's AAV5 gene therapies has experienced any T-cell-mediated immune response to the capsid.

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 potential safety or efficacy of AMT-061, the potential for AMT-061 to be a life-changing treatment for patients or to be considered a best-in-class gene therapy or a major advancement in gene therapy, the expected timing of the Phase III HOPE-B pivotal trial or the Phase IIb dose-confirmation study of AMT-061, and the expected announcement of data from any trial or study. 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, the results of any clinical trial or study, the timing of any clinical trial or study, collaboration arrangements, corporate reorganizations and strategic shifts, manufacturing issues, regulatory oversight, 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 April 30, 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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