uniQure Announces Clinical Update on Hemophilia B Gene Therapy Program

~ Company to hold conference call today at 8:30 a.m. ET ~

Lexington, MA and Amsterdam, the Netherlands, December 21, 2020 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that its hemophilia B gene therapy program, including the pivotal, Phase III HOPE-B study, has been placed on clinical hold by the U.S. Food and Drug Administration (FDA). Patient dosing is complete in each of uniQure's three hemophilia B gene therapy studies, and there is no plan to enroll or treat additional patients.

The clinical hold was initiated following the submission of a safety report in mid-December relating to a possibly related serious adverse event associated with a preliminary diagnosis of hepatocellular carcinoma (HCC), a form of liver cancer, in one patient in the HOPE-B trial that was treated with etranacogene dezaparvovec (AMT-061) in October 2019. The patient has multiple risk factors associated with HCC, including a twenty-five-year history of hepatitis C (HCV), hepatitis B virus (HBV), evidence of non-alcoholic fatty liver disease and advanced age. Chronic infections with hepatitis B and C have been associated with approximately 80% of HCC cases.[1]

The liver lesion was detected during a routine abdominal ultrasound conducted as part of the required study assessments in patients at one-year post dosing. A full surgical resection of the lesion is scheduled this week that will allow for confirmation of the diagnosis. No other cases of HCC have been reported in uniQure clinical trials conducted in more than 100 patients in hemophilia B and other indications, with some patients dosed more than 10 years ago.

"Patient safety will always be our top priority, and we are working closely with the FDA and our advisors to conduct a thorough investigation into the cause of this event which we expect to be completed in early 2021," said Ricardo Dolmetsch, Ph.D., president of research and development at uniQure. "We will investigate whether there is a relationship to treatment. At this time, we do not have adequate data to determine a possible causal relationship, especially in the context of the other known risk factors."

"All patients in our hemophilia B gene therapy program, including the 54 patients in HOPE-B, will continue to be monitored by their care teams while we gather additional information as rapidly as possible," stated Matt Kapusta, chief executive officer. "We do not anticipate any impact to our regulatory submission timeline for the hemophilia B program as a result of this clinical hold."

Investor Conference Call Information

[1] El-Serag HB. Epidemiology of viral hepatitis and hepatocellular carcinoma. Gastroenterology. 2012 May;142(6):1264-1273.e1. doi: 10.1053/j.gastro.2011.12.061. PMID: 22537432; PMCID: PMC3338949.

uniQure management along with Dr. Steven Pipe, principal investigator in the pivotal, Phase III HOPE-B trial, and Dr. Graham Foster, professor of hepatology at Queen Mary University of London, UK, will host a conference call today, Monday, December 21, 2020, at 8:30 a.m. ET. The conference call may be accessed by dialing (877) 870–9135 for domestic callers and +44 020 719 283 38 for international callers. The passcode is 5892217. Please specify to the operator that you would like to join the "uniQure Conference Call." The webcast may also be accessed through the Investors section of the uniQure website at http://uniqure.com/investors-newsroom/overview.php. Following the live webcast, a replay of the call will be archived for several weeks.

About Etranacogene Dezaparvovec

Etranacogene dezaparvovec consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). uniQure holds multiple issued patents in the United States and Canada broadly covering methods of treating bleeding disorders, including hemophilia B, using AAV gene therapy with the FIX-Padua variant. Etranacogene dezaparvovec has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration and access to Priority Medicine (PRIME) regulatory initiative by the European Medicines Agency. In June 2020, the Company and CSL Behring entered into a licensing agreement providing CSL Behring with exclusive global rights to etranacogene dezaparvovec. This licensing agreement is subject to antitrust regulatory review in the United States, Australia and the United Kingdom that is currently ongoing.

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 gene therapies to treat patients with hemophilia B, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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, whether clinical data from the HOPE-B Phase III pivotal trial will be included in regulatory submissions to the FDA and EMA in the second half of 2021 or ever, whether the investigation into the cause of the event that resulted in a clinical hold will be completed in early 2021, whether there will be any impact to our regulatory submission timeline for the hemophilia B program as a result of this clinical hold, and whether we will obtain regulatory approval of our agreement with CSL Behring or otherwise close the transaction. 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, clinical results, 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 October 27, 2020. 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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