uniQure Announces License Agreement with CSL Behring to Commercialize Hemophilia B Gene Therapy

~ CSL Behring Obtains Exclusive Global Rights to Develop and Commercialize uniQure's Differentiated Gene Therapy Candidate for Hemophilia B ~

~ uniQure Eligible to Receive More Than $2 Billion, Including $450 Million in Upfront Cash, $1.6 Billion in Milestone Payments, and Double-Digit Royalties Ranging Up to a Low-Twenties Percentage of Net Sales ~

~ Agreement Leverages CSL Behring's Global Hematology Capabilities and Infrastructure to Benefit Hemophilia B Patients Worldwide ~

~ Transaction Expected to Enable uniQure to Strategically Expand and Accelerate Pipeline and Platform ~

~ uniQure to Host Conference Call Today, June 24, 2020, at 5:30 p.m. EDT ~

Lexington, MA and Amsterdam, the Netherlands, June 24, 2020 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that uniQure and CSL Behring have entered into a licensing agreement providing CSL Behring with exclusive global rights to etranacogene dezaparvovec, uniQure's investigational gene therapy for patients with hemophilia B. Etranacogene dezaparvovec consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). Under the terms of the agreement, uniQure will receive a $450 million upfront cash payment and be eligible to receive up to $1.6 billion in payments based on regulatory and commercial milestones. uniQure will also be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percentage of net product sales arising from the collaboration.

The collaboration leverages CSL Behring's strong global reach and commercial infrastructure in hematology to accelerate access of etranacogene dezaparvovec to hemophilia B patients around the world.

"We are thrilled to enter into this commercialization and license agreement with CSL Behring, an ideal commercial partner with global reach and decades of expertise in hemophilia," stated Matt Kapusta, chief executive officer of uniQure. "We believe that through this arrangement, we are ideally positioned to deliver globally our innovative gene therapy to the largest number of hemophilia B patients as quickly as possible."

"The transaction represents a major milestone in the development of etranacogene dezaparvovec and, when closed, we expect that it will provide uniQure with significant financial resources to advance and expand our pipeline of gene therapy candidates, anchored by AMT-130 in Huntington's disease, and to invest further in our leading gene therapy manufacturing and technology platform to support pipeline growth," he added.

As a CSL Limited (ASX:CSL;USOTC:CSLLY) company, CSL Behring is a global biotherapeutics leader delivering lifesaving medicines to patients with rare and serious diseases. A global leader in treating bleeding disorders, CSL Behring has been delivering innovations for the hemophilia patient community for more than 30 years. The company reported more than $1 billion in sales of hemophilia-related medicines in 2019.

"Our vision with hemophilia B patients is to offer transformational treatment paradigms that help free them from the lifelong burden of this disease," said CSL's CEO and Managing Director Paul Perreault. "With more than three decades of providing lifesaving innovations for the global bleeding disorders community, we are well positioned to maximize the potential benefit of this therapy. Upon approval, we believe this next-generation therapy will be highly complementary to our existing best-in-class hemophilia B product portfolio with an alternate best-in-class treatment option."

Under the terms of the agreement, uniQure will be responsible for the completion of the HOPE-B pivotal study, manufacturing process validation, and the manufacturing supply of etranacogene dezaparvovec until such time that these capabilities are transferred to CSL Behring. Clinical development and regulatory activities performed by uniQure under the agreement will be reimbursed by CSL Behring. CSL Behring will be responsible for regulatory submissions and commercialization of etranacogene dezaparvovec.

The closing of the transaction is contingent on completion of review under antitrust laws in the United States, Australia and the United Kingdom.

Accelerate Build-out of Innovative Gene Therapy Pipeline and Platform

uniQure expects that the agreement will provide additional capital to significantly accelerate and expand its pipeline of innovative gene therapies, including advancing the Phase I/II study of AMT-130 in Huntington's disease, initiating IND-enabling studies of AMT-150 in spinocerebellar ataxia type 3, selecting a lead candidate in Fabry disease and progressing other current and new candidates for central nervous system disorders and rare liver-directed diseases. Regarding AMT-130, uniQure recently announced the successful completion of the first two patient procedures in the Phase I/II study and anticipates announcing early safety data in the second half of 2020 and initial efficacy data in 2021.

uniQure plans to continue to leverage its leading gene therapy platform, including the Company's deep expertise with AAV5, to develop potentially best-in-class gene therapies. AAV5-based gene therapies have been demonstrated to be safe and well tolerated in a multitude of clinical trials, including uniQure trials conducted in hemophilia B and other indications. No patient treated in clinical trials with uniQure's AAV5 gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be viable treatments in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment. uniQure also may seek to in-license or acquire additional product candidates that align with its research and development strategy.

In addition, uniQure plans to further strengthen its proprietary gene therapy platform by expanding its manufacturing capacity to support a broad pipeline, including product candidates for diseases with larger prevalence, as well as investing further in new technologies to improve the efficacy, safety and applicability of its gene therapies to patients.

As part of uniQure's effort to focus on those gene therapy programs that have the greatest potential to improve patients' lives and generate long-term value for shareholders, uniQure plans to de-prioritize its research program of AMT-180 for patients with hemophilia A.

Moelis & Company acted as a financial advisor to uniQure in this transaction.

Conference Call Today at 5:30 p.m. EDT

uniQure will host a conference call today, June 24, 2020, at 5:30 p.m. Eastern Daylight Time. 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 for the call is 9499239. Please specify to the operator that you would like to join the "uniQure Conference Call." The conference call will be webcast live under the investor relations section of uniQure's website at www.uniQure.com and will be archived there following the call for 90 days.

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 the parties will successfully complete the review under applicable antitrust laws *or otherwise close the transaction, whether uniQure will receive the upfront cash payment or any of the financial benefits of the agreement; whether the collaboration will benefit Hemophilia B patients worldwide, whether the parties to the agreement will establish a new standard of care for patients with hemophilia B, whether uniQure will be able to accelerate or expand its pipeline of innovative gene therapies or its technology platform, including advancing the Phase I/II study of AMT-130 in Huntington's disease, initiating IND-enabling studies of AMT-150 in spinocerebellar ataxia type 3, selecting a lead product candidate for Fabry disease, or progressing current or additional candidates for central nervous system disorders and other genetic diseases, whether uniQure will announce early safety data from its Phase I/II study of AMT-130 in the second half of 2020 and initial efficacy data in 2021 or ever, whether uniQure will develop best-in-class gene therapies, whether uniQure will in-license or acquire additional product candidates, whether uniQure will expand its manufacturing capacity to support a broad pipeline, such as product candidates for diseases with larger prevalence, and whether uniQure will obtain enabling technologies that improve the efficacy or safety of its gene therapies. uniQure's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our clinical development activities, clinical results, collaboration arrangements, 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 Annual Report on Form 10-Q filed on April 29, 2020. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and uniQure assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.*

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