uniQure and CSL Behring Announce Primary Endpoint Achieved in HOPE-B Pivotal Trial of Etranacogene Dezaparvovec Gene Therapy in Patients with Hemophilia B

~ Largest gene therapy study in hemophilia B achieved primary endpoint of non-inferiority in annualized bleeding rate after stable Factor IX (FIX) expression, assessed at 18 months following a single dose of etranacogene dezaparvovec ~

~ Etranacogene dezaparvovec also achieved secondary endpoint demonstrating statistical superiority in reduction of annualized bleeding rate compared to baseline FIX prophylactic therapy ~

~ Stable and durable FIX levels with mean FIX activity of 36.9 percent of normal in full study population at 18-months, compared to a mean of 39.0 percent of normal at 6 months ~

~ Manufacturing operations supporting process validation of etranacogene dezaparvovec successfully completed by uniQure ~

Lexington, MA and Amsterdam, the Netherlands, King of Prussia, PA , December 9, 2021 — CSL Behring, a global biotherapeutics leader, and uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that etranacogene dezaparvovec, an investigational adeno-associated virus five (AAV5)-based gene therapy for the treatment of patients with severe to moderately severe hemophilia B, achieved the pre-specified primary endpoint of non-inferiority in annualized bleeding rate (ABR) 18-months following administration compared to baseline Factor IX (FIX) prophylactic therapy in the pivotal Phase III HOPE-B gene therapy trial. The study also successfully achieved a secondary endpoint demonstrating statistical superiority in reduction of ABR compared to baseline FIX prophylactic therapy.

The primary endpoint in the pivotal study was 52-week ABR after achievement of stable FIX expression compared with the six-month lead-in period, considering all bleeds regardless of investigator adjudication as true bleeds. For this endpoint, ABR was measured from month seven to month 18 after infusion, ensuring the observation period represented likely steady-state FIX transgene expression. Secondary endpoints included assessment of FIX activity and statistical superiority of ABR after dosing.

“We are very pleased with these top-line results from what is the largest and first pivotal trial of a gene therapy for patients with hemophilia B,” stated Ricardo Dolmetsch, Ph.D., president of research and development at uniQure. “The HOPE-B data not only achieved the pre-specified primary endpoint of non-inferiority in annualized bleeding rate following 12 months or more of stable FIX expression, but also the secondary endpoint of superiority in reduction of annualized bleeding, while continuing to demonstrate durability and stability in FIX levels and other benefits to this point in the study.”

“On behalf of uniQure, we extend our heartfelt gratitude to all the HOPE-B clinical trial patients and their families, as well as the trial investigators,” he continued. “We now look forward to collaborating with CSL Behring on completing the regulatory submissions that we hope will advance etranacogene dezaparvovec one step closer to reaching hemophilia B patients around the world.”

uniQure led the multi-year clinical development of etranacogene dezaparvovec prior to entering into a Commercialization and License Agreement with CSL Behring in June 2020 for exclusive global rights to etranacogene dezaparvovec. Earlier this month, uniQure successfully completed manufacturing operations supporting process validation of etranacogene dezaparvovec.

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. CSL Behring plans to submit regulatory applications for marketing approval of etranacogene dezaparvovec in the United States and European Union in the first half of 2022.

Top-line Data Results

A total of 54 patients received a single dose of etranacogene dezaparvovec in the pivotal trial, with 53 patients completing at least 18 months of follow-up

ABR for all bleeds after stable FIX expression, assessed at 18 months, was 1.51 compared with the ABR of 4.19 for the lead-in period of at least six months, achieving the primary non-inferiority endpoint and a secondary superiority endpoint ($p=0.0002$) in the HOPE-B trial. ABR for investigator-adjudicated FIX-treated bleeds was 0.83 compared with lead-in ABR of 3.65 ($p<0.0001$).

Data from the HOPE-B pivotal trial showed that patients continued to demonstrate durable, sustained increases in FIX activity at 18 months post-infusion with a mean FIX activity of 36.9 percent of normal as measured by a one-stage APTT-based clotting assay, compared to mean FIX activity of 39.0 percent of normal at six months post-infusion.

Etranacogene dezaparvovec was generally well-tolerated with over 80% of adverse events considered mild. One death resulting from urosepsis and cardiogenic shock in a 77-year-old patient at 65-weeks following dosing was considered unrelated to treatment by investigators and the company sponsor. A serious adverse event of hepatocellular carcinoma (HCC) was identified in one patient. Independent molecular characterization and vector integration analysis of the HCC and adjacent tissue supported the conclusion by the investigator and company sponsor that the HCC was unrelated to treatment with etranacogene dezaparvovec. No inhibitors to FIX were reported.

“These encouraging results illustrate the potential that gene therapy has to be a long-term treatment option for patients living with hemophilia B and we look forward to sharing more detailed data with the medical community in the near future,” stated Brahm Goldstein, MD, MCR, Vice President, Research and Development, Hematology at CSL Behring. “This milestone advances our efforts towards expected regulatory submissions in first half of 2022.”

HOPE-B Pivotal Trial Design

The pivotal Phase III HOPE-B trial is a multinational, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec. Fifty-four adult hemophilia B patients classified as severe or moderately severe (defined as less than or equal to 2% of normal FIX activity) and requiring prophylactic FIX replacement therapy were enrolled in a prospective, six-month observational period during which time they continued to use their current standard of care therapy to establish a baseline annualized bleeding rate. No prophylactic immunosuppression was provided to patients upon entering the study. After the six-month lead-in period, patients received a single intravenous administration of etranacogene dezaparvovec at the 2x10^13 gc/kg dose. Patients were not excluded

from the trial based on pre-existing neutralizing antibodies (NAbs) to AAV5. Forty-three percent of patients in the study had pre-existing NAbs to AAV5 up to a maximum observed pre-dosing titer of over 3,200.

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 temporal lobe epilepsy, Alzheimer's, Parkinson's and ALS. www.uniQure.com

About CSL Behring
CSL Behring is a global biotherapeutics leader driven by our promise to save lives. Focused on serving patients' needs by using the latest technologies, we discover, develop and deliver innovative therapies for people living with conditions in the immunology, hematology, cardiovascular and metabolic, respiratory, and transplant therapeutic areas. We use three strategic scientific platforms of plasma fractionation, recombinant protein technology, and cell and gene therapy to support continued innovation and continually refine ways in which products can address unmet medical needs and help patients lead full lives.

CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. Our parent company, CSL Limited (ASX:CSL; USOTC:CSLLY), headquartered in Melbourne, Australia, employs more than 25,000 people, and delivers its lifesaving therapies to people in more than 100 countries. For inspiring stories about the promise of biotechnology, visit CSLBehring.com/Vita and follow us on Twitter.com/CSLBehring.

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 CSL Behring will submit a BLA for etranacogene dezaparvovec in the first half of 2022, whether etranacogene dezaparvovec will reach hemophilia B patients around the world, and whether etranacogene dezaparvovec has the potential to provide well-tolerated, long-term clinical benefits, and whether AAV5-based gene therapies can provide clinical benefit to patients with pre-existing neutralizing antibodies. Our 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 Commercialization and License Agreement with CSL Behring, 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 periodic securities filings, including its Annual Report on Form 10-K filed March 2, 2020 and Quarterly Report on Form 10-Q filed on October 25, 2021. 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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