



uniQure Presents HOPE-B Clinical Data Demonstrating Clinical Benefit in Hemophilia B Patients with Pre-existing Antibodies to AAV5 Vector

May 13, 2021

~ Data Show No Clinically Significant Correlation Between Pre-Existing NABs and FIX Activity ~

~ Further Supports Potential for AAV5 Gene Therapies to be Viable Treatments in Nearly All Patients ~

~ Data Presented at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting ~

LEXINGTON, Mass. and AMSTERDAM, May 13, 2021 (GLOBE NEWSWIRE) -- [uniQure N.V.](https://www.uniqure.com) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, yesterday presented 26-week clinical data from the pivotal, Phase III HOPE-B gene therapy trial of [etranacogene dezaparvovec](#) showing clinical benefit in hemophilia B patients with pre-existing neutralizing antibodies (NABs) to AAV5, and no relationship between AAV5 NABs and the tolerability of the therapy.

The data were featured on Wednesday, May 12, 2021 at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting in an oral presentation by Michael Recht, M.D., Ph.D., M.B.A., professor of pediatrics, division of hematology and oncology at the Oregon Health & Science University School of Medicine. These data were previously presented at a medical meeting late last year.

"The ASGCT presentation highlights these initial HOPE-B data that demonstrate the successful treatment with an AAV5 gene therapy of patients with pre-existing NABs," stated [Ricardo Dolmetsch](#), Ph.D., president of Research and Development at uniQure. "Patients in the trial who may not have been eligible for other gene therapies because of pre-existing neutralizing antibodies have achieved similar results with etranacogene dezaparvovec compared to those who did not have pre-existing NABs. We believe this distinguishes etranacogene dezaparvovec as the only hemophilia gene therapy shown in a clinical trial to have the potential to treat nearly all patients, regardless of NAB levels in the generally prevalent range."

Patients in the Phase III HOPE-B clinical study were initially enrolled into a prospective, observational lead-in period of at least six months during which bleeding events and FIX replacement therapy usage were monitored. All patients required prophylactic routine FIX replacement prior to entering the clinical trial, and patients were not excluded from the trial based on pre-existing NABs to AAV5.

Fifty-four participants were dosed and completed 26 weeks of follow-up, 31 of whom (57.4%) had no pre-existing NABs to AAV5. Of the 23 participants (42.6%) with pre-existing NABs, the median titer was 56.9, with a distribution representative of the general population.

No clinically significant correlation of pre-existing NABs with FIX activity was observed up to a titer of 678, a range expected to include more than 95 percent of the general population. Mean FIX activity at 26 weeks was 32.7 percent in participants with NABs versus 41.3 percent in those without. A single participant with a NAB titer of 3,212 did not respond and remained on prophylaxis; it is expected that less than 1 percent of the general population have a pre-existing NAB titer of more than 3,000. All other participants, both with and without NABs, discontinued prophylaxis and remained prophylaxis-free at 26 weeks.

The data presentation from ASGCT can be found on the [Investors & Newsroom section](#) of uniQure's corporate website.

About Etranacogene Dezaparvovec

[Etranacogene dezaparvovec](#) (AMT-061) is an investigational adeno-associated virus five (AAV5)-based gene therapy for the treatment of patients with severe and moderately severe 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). uniQure and CSL Behring have entered into a commercialization and license agreement providing CSL Behring exclusive global commercialization rights to etranacogene dezaparvovec. The collaboration combines uniQure's differentiated gene therapy candidate in hemophilia B and CSL Behring's strong global reach and commercial infrastructure in hematology in an effort to accelerate access of etranacogene dezaparvovec to hemophilia B patients around the world.

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 etranacogene dezaparvovec or AAV5-based gene therapies generally can provide clinical benefit to patients with pre-existing neutralizing antibodies. 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 Commercialization and License Agreement with CSL Behring, 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 Quarterly Report on Form 10-Q filed on May 10, 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.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor

Direct: 339-970-7536

Chiara Russo

Direct: 617-306-9137

FOR MEDIA:

Tom Malone

Direct: 339-970-7558

Mobile: 617-680-9452
m.cantor@uniQure.com

Mobile: 617-306-9137
c.russo@uniQure.com

Mobile: 339-223-8541
t.malone@uniQure.com