



uniQure Announces Publication of Positive Interim Data from Phase IIb Clinical Trial of Etranacogene Dezaparvovec

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LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Oct. 31, 2019 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today announced the publication of 26-week interim data from the ongoing Phase IIb clinical trial of [etranacogene dezaparvovec](#), an investigational gene therapy for hemophilia B, in the journal, *Blood Advances*.

With a combined follow-up of 78 weeks of observation on the three adult male participants with severe hemophilia B, the mean Factor IX (FIX) activity was 47 percent of normal (range of 33 percent to 57 percent of normal) following a single administration of etranacogene dezaparvovec (AMT-061). Consistent with the observed Factor IX activity, the treatment was associated with a complete cessation of bleeds up to the 26-week interim endpoint, and no patient in the study required any infusions of FIX replacement therapy during this time.

In the year prior to administration of etranacogene dezaparvovec, all three participants received prophylactic FIX replacement, as well as additional doses of FIX to treat bleeding events. Medical records showed that the 3 participants experienced a total of 9 bleeds in the year prior to treatment despite routine FIX prophylaxis.

Etranacogene dezaparvovec was generally well tolerated, with no clinically significant elevations of liver enzymes or inflammatory markers observed, and no use of corticosteroids related to treatment required.

"In individuals with severe and moderately severe hemophilia B, etranacogene dezaparvovec resulted in clinically-relevant increases in FIX activity, cessation of bleeds and abrogated the need for FIX replacement, despite the presence of pre-existing anti-AAV5 neutralizing antibodies detected by the highly sensitive luciferase assay," 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 an investigator in the Phase IIb clinical trial. "While these observations are limited to three participants, the consistency of results among participants supports the ongoing pivotal Phase III HOPE B trial which will further assess the safety and efficacy of etranacogene dezaparvovec. If these results are confirmed by the Phase III trial, physicians will be provided with increased confidence that reliable clinical outcomes can be achieved following gene transfer."

"This publication supports the potential of etranacogene dezaparvovec to substantially improve the quality of life for hemophilia B patients through a one-time administration that results in sustained Factor IX activity and may result in a cessation of bleeding episodes," stated [Robert Gut](#), M.D., Ph.D., chief medical officer at uniQure and co-author of the paper. "We are pleased to have these interim safety and efficacy data of etranacogene dezaparvovec published in a peer-reviewed journal and look forward to sharing top-line data from the pivotal Phase III HOPE-B study in 2020."

The Company has completed patient enrollment in the HOPE-B pivotal trial and expects top-line data by the end of next year.

"Etranacogene dezaparvovec (AMT-061 Phase 2b): Normal/Near Normal FIX Activity and Bleed Cessation in Hemophilia B," is available on-line in the journal, *Blood Advances*.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec, also known as AMT-061, consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). AAV5-based gene therapies have been demonstrated to be safe and well tolerated in many clinical trials, including four uniQure trials conducted in 25 patients in hemophilia B and other indications. No patient treated in clinical trials with the Company's AAV5-based 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 clinically effective in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

About the Pivotal Phase III HOPE-B Study

The pivotal Phase III HOPE-B trial is a multinational, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec. Adult hemophilia B patients classified as severe or moderately severe are 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 etranacogene dezaparvovec at the 2×10^{13} gc/kg dose. Dosing of patients in the HOPE-B pivotal trial was initiated in January 2019.

The study's primary endpoint is the assessment of Factor IX activity 26 weeks after dosing. Secondary endpoints include annualized bleeding rate (ABR) and usage of Factor IX replacement therapy over a 52-week time frame, as well as other efficacy and safety aspects. Post-treatment, patients will be followed for 5 years.

Patients enrolled in the HOPE-B pivotal 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-based gene therapies may be viable treatments for at least 97% of patients.

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, hemophilia A, 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, the ability of etranacogene dezaparvovec to substantially improve the quality of life for hemophilia B patients through a one-time administration or to result in sustained Factor IX activity or to result in a cessation of bleeding episodes, whether the Phase IIb or Phase III trial data or any data will provide an increased confidence that reliable clinical outcomes can be achieved following gene transfer, and whether top-line data from the Phase III trial can be shared in 2020 or ever. 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 July 29, 2019. 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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