



uniQure Presents New Clinical Data in Hemophilia B Patients Demonstrating Therapeutic Efficacy of AAV5 Gene Therapy in the Presence of Pre-Existing Neutralizing Antibodies

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-- Findings Further Support Expanding the Eligibility of AAV5 Gene Therapies to Nearly All Patients with Hemophilia B --

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, July 11, 2017 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today presented new clinical data demonstrating that the presence of pre-existing anti-AAV5 neutralizing antibodies (NABs) does not predict the potential efficacy of AAV5-mediated gene transfer in patients with hemophilia B. Clinically meaningful factor IX (FIX) activity levels from the ongoing Phase I-II trial of AMT-060 were observed at NAB titers up to 1:341, determined as corresponding up to the 90th percentile of a healthy control population. NABs were quantified in the blood sera of these patients using a highly sensitive assay. These clinical data were presented today in a poster presentation at the 26th Biennial Congress of the International Society on Thrombosis and Hemostasis (ISTH), taking place this week in Berlin, Germany.

The presence of pre-existing NABs to adeno-associated virus (AAV) vectors has long posed a critical challenge for the clinical application of gene therapies, as patients who currently screen positive for NABs are generally excluded from treatment. Researchers from uniQure recently presented data in non-human primates suggesting that AAV5 could successfully mediate gene transfer in the presence of NABs at levels as high as 1:1031.

In a poster presentation at the ISTH meeting, a re-analysis was described of pre-gene transfer screening samples from the 10 patients who have been treated in the ongoing Phase I/II trial of AMT-060 for hemophilia B. The patients had tested negative for pre-existing anti-AAV5 NABs using a green fluorescent protein-based (GFP) assay before receiving treatment. These samples were later re-assessed using a highly sensitive luciferase-based (LUC) NAB assay. Anti-AAV5 NABs were detected retrospectively in three patients who had been treated with the low dose (5×10^{12} gc/kg) of AMT-060. However, all three patients presented increases in FIX expression and, especially, the patient with the highest NAB level (titer 1:341) had the highest FIX-activity (steady-state FIX 6.8% of normal; latest FIX measurement 10.7% of normal) among all five patients treated in the low-dose cohort. None of the three patients who tested positive for NAB titers, experienced over time elevations in liver enzymes post gene transfer, FIX activity loss, or clinically relevant T-cell responses to the capsid.

"These clinical data show that hemophilia B patients presenting with neutralizing antibodies may be considered eligible for AAV5-mediated gene transfer," stated Matthew Kapusta, chief executive officer at uniQure. "This development potentially expands the applicability of AAV5 gene therapies to nearly all hemophilia B patients. We believe these factors contribute to making AAV5 a potential best-in-class vector for delivering gene therapies more effectively and safely to a greater portion of patients in need of treatment."

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 and partnered gene therapies to treat patients with hemophilia, Huntington's disease and cardiovascular 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, statements regarding the development of our gene therapy product candidates, including the future development of AMT-060, the success of our collaborations and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with corporate reorganizations and strategic shifts, collaboration arrangements, our and our collaborators' clinical development activities, 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 2016 Annual Report on Form 10-K filed on March 15, 2017. 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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