



uniQure Publishes Data Demonstrating Wide Distribution of AAV5 in the Central Nervous System

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Study published in Gene Therapy will help guide clinical development of gene therapy product candidate AMT-130 in Huntington's disease

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, April 04, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the online publication in *Gene Therapy* of data demonstrating widespread transduction in the CNS following direct injection of uniQure's AAV5 vector in a large animal model. uniQure has exclusive, worldwide rights to AAV5 for use in therapeutic products delivered to the brain or liver.

The method of injection used was found to result in very controlled and accurate administration with no adverse events observed in the non-human primates. Varying doses of AAV5 achieved predictable transduction of connected areas of the brain. The data demonstrate that AAV5 is a very effective vector for the central nervous system and has potential for the treatment of a wide range of neurological pathologies. These data will help guide the clinical development of uniQure's gene therapy product candidate AMT-130, which consists of an AAV5 vector carrying an artificial micro-RNA that silences the huntingtin gene for the treatment of Huntington's disease.

In the publication entitled "MR-guided parenchymal delivery of adeno-associated viral vector serotype 5 in nonhuman primate brain," uniQure researchers in collaboration with experts from the University of California, San Francisco, describe the predictable and dose-dependent axonal transport of AAV5 after MRI-guided, convection-enhanced, parenchymal infusion. The results indicate that AAV5 can effectively achieve wide distribution throughout the central nervous system, which is crucial for the treatment of many CNS disorders, such as Huntington's disease.

"This study further supports our belief that AAV5 is an optimal vector for CNS-delivery of gene therapy, and is an important step forward in defining the parameters for direct injection of AAV5 into the brain for future clinical applications in humans," stated Harald Petry, uniQure's chief scientific officer. "In particular, these data will help guide the design and development of the planned clinical trials of AMT-130. We look forward to making several preclinical data presentations at scientific meetings this year and to file an Investigational New Drug (IND) application for AMT-130 in 2018."

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 candidate AMT-130. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with 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 with the Securities and Exchange Commission 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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