uniQure Announces the Successful Development and Scale-up of Manufacturing Processes for Its Hemophilia B Gene Therapy Program

July 21, 2017

-- Commercial-Scale Batches of AMT-060 Successfully Produced in Accordance with Good Manufacturing Practices in Lexington, MA Facility --

-- Manufacturing Process to be Leveraged in Huntington's Program, With Production Expected to Begin Before Year End --

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, July 21, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (Nasdaq:QURE), a leader in human gene therapy and AAV manufacturing, today announced that it has successfully developed and optimized a reproducible and scaled-up manufacturing process for producing its lead gene therapy candidate for patients with hemophilia B at its state-of-the-art manufacturing facility in Lexington, Massachusetts. This progress includes the validation of analytical methods required to manufacture and test product in accordance with Good Manufacturing Practices (GMP). The Company has also finalized its comparability protocol and is making progress on its execution.

"We have now achieved the successful manufacturing of AMT-060 at our Lexington facility at a scale sufficient to support pivotal clinical trials and commercial supply," stated Matt Kapusta, chief executive officer of uniQure. "We have made significant progress over the past several months to now be in a position where we have developed a commercial-scale process and are evaluating our completed batches to assess comparability. We look forward to finalizing this work in anticipation of meetings with regulators to further discuss plans to advance our hemophilia B program into a pivotal study next year."

As a result of this progress, the Company expects to meet with the U.S. Food and Drug Administration and European Medicines Agency in the early fall. At the same time, uniQure expects to leverage its fully developed, commercial-scale, manufacturing process to begin producing GMP material associated with a Phase I/II trial of AMT-130 in Huntington's disease by the end of the year.

About Gene Therapy Manufacturing at uniQure

uniQure's facility in Lexington, Massachusetts is one of the largest, most versatile gene therapy manufacturing plants in the world. uniQure made significant investments in designing, constructing and equipping the 55,000-square foot facility with state-of-the-art laboratories and commercial-scale, GMP production capabilities to support all of its existing programs, with flexibility to expand further.

uniQure produces its AAV-based gene therapies in its own facilities with its proprietary manufacturing process. In aggregate, uniQure owns seven AAV manufacturing patent families in which 130 patents have been granted worldwide, and 55 applications are pending, including in the U.S., Europe, Asia and South America. A comprehensive summary of uniQure's AAV manufacturing technology is available on the Company's website.

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 CNS, liver/metabolic 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, the development and manufacture of our gene therapy product candidates, 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 Quarterly Report on Form 10-Q filed on May 9, 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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