uniQure Announces Featured Presentations of New Data on Spinocerebellar Ataxia Type 3 at the 2019 American Academy of Neurology Annual Meeting

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~ Data Show AMT-150 Able to Significantly Lower Mutant Ataxin-3 Protein in SCA3 Disease Model ~

~ Provides Further Support of Proof-of-concept of Company's Proprietary miQURE™ Gene Silencing Platform ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, May 07, 2019 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, will today present preclinical data on its gene therapy candidate, AMT-150, for the treatment of Spinocerebellar Ataxia 3 (SCA3). The abstract entitled, "Development of an AAV-based microRNA Gene Therapy for Treating Spinocerebellar Ataxia Type 3," is being recognized by the American Academy of Neurology for dual oral and poster presentations during its annual meeting taking place this week in Philadelphia, PA.

Spinocerebellar Ataxia Type 3, also known as Machado-Joseph disease, is caused by a CAG-repeat expansion in the ATXN3 gene that results in an abnormal form of the toxic protein ataxin-3, leading to brain degeneration that results in movement disorders, rigidity, muscular atrophy and paralysis. There are no disease-modifying treatments for patients with SCA3, or medications to slow the progressive course of the fatal disease.

uniQure's AMT-150 incorporates the Company's proprietary miQURE™ gene silencing technology and comprises an AAV5 vector to deliver a microRNA that is designed to halt ataxia in early manifest SCA3 patients. AMT-150 is delivered by intra-cisterna magna injection into the cerebrospinal fluid.

AMT-150 Preclinical Data Findings:

- Mechanistic proof-of-concept of the non-allele-specific ataxin-3 protein-silencing approach was demonstrated using artificial microRNA candidates engineered to target the ataxin-3 gene in a SCA3 knock-in mouse model. The 6-week proof-of-concept study demonstrated that a single AMT-150 injection in the cerebrospinal fluid resulted in strong AAV transduction and significant mutant ataxin-3 lowering for at least one microRNA candidate at each of the primary sites of disease neuropathology, namely the cerebellum (up to 53%) and brainstem (up to 65%).

- These results were corroborated by preclinical studies in human induced Pluripotent Stem Cell (iPSC)-derived neurons showing a dose-dependent lowering of ataxin-3 mRNA of up to 55%.

- These studies, along with our previously-reported data in Huntington's disease, further demonstrate the potential utility and safety profile of the miQURE™ technology, the Company's proprietary gene-silencing platform.

"We believe that the data from these preclinical studies in the knock-in mouse model and in iPSC-derived neurons show the potential of AMT-150 to alter the course of this devastating disease after a single administration," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "We are very proud of our proprietary, in-house developed miQURE™ technology, and we believe that it has the potential to treat a wide range of polyglutamine diseases, including Huntington's disease and SCA3. We will continue to conduct additional research on AMT-150 to advance it toward our goal of IND-enabling studies."

AMT-130 Data Presentations

In addition to the highlighted presentations on AMT-150 in SCA3, two encore presentations on the development of AMT-130 in Huntington's disease also were featured during the AAN conference. One presentation included findings from magnetic resonance imaging volumetric analysis in twenty early manifest Huntington's disease patients to determine the safety of delivering gene therapy to the striatum, or deep structures of the brain. The second reported on the biodistribution and tolerability of AMT-130 after bilateral intra-striatal delivery to non-human primates.

An overview of the data presented at AAN can be found in the Investor section of uniQure's corporate 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 hemophilia, Huntington's disease and other severe genetic 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 risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies, our ability to advance our pipeline programs in SCA3 and Huntington's disease, our ability to move closer to providing potentially transformative therapies to patients and further demonstrate the importance of our industry leading technology platform and AAV manufacturing capabilities, and/or the development and regulatory approval of our

product candidates in the United States or in Europe, whether AMT-150 proves to alter the course of this devastating disease after a single administration or at all, whether our miQURE™ technology is able to treat SCA3, Huntington's disease or a wide range of polyglutamine diseases, and whether we will be able to complete IND-enabling studies. 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 April 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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