



uniQure Strengthens Intellectual Property Portfolio with Acquisition of Patent Family Providing Broad Protection of the Hyperactive Padua Variant of Factor IX (FIX-Padua)

~ Issued Patent in the U.S. Covers Use of FIX-Padua in Gene Therapy ~

~ Divisional patent applications filed to further strengthen Padua intellectual property position ~

Lexington, MA and Amsterdam, the Netherlands, October 19, 2017 — [uniQure N.V.](http://www.uniqure.com) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that the Company has acquired a patent family, with claims issued in the U.S., that broadly covers a hyperactive variant of Factor IX carrying an R338L mutation (often referred to as “FIX-Padua”) and its use in gene therapy for the treatment of coagulopathies, including hemophilia B. This patent family was acquired from the inventor, Professor Paolo Simioni, a renowned hemophilia expert at the University of Padua, Italy, who is widely recognized as the first to identify this mutant.

Professor Simioni filed a PCT application on September 15, 2009, and patent applications are pending in the U.S., Europe, and Canada. The U.S. Patent and Trademark Office issued U.S. Patent 9,249,405 on February 2, 2016, which includes claims directed to Factor IX protein with a leucine at the R338 position of the protein sequence, nucleic acid sequences coding for this protein, and therapeutic applications, including gene therapy. Additional fast track divisional patent applications have also been filed in the U.S. and in Europe that would further strengthen uniQure’s intellectual property position.

“I have worked my entire career in the field of coagulopathies, and in my experience the innovation of FIX-Padua holds great promise for the treatment of hemophilia B,” said Professor Simioni. “Combined with the known safety profile of the AAV5 vector, this gene has the potential to significantly improve the health of hemophilia B patients. I look forward to working with uniQure as they advance what I believe will be the most effective gene therapy for patients suffering from hemophilia B.”

Professor Simioni is serving as advisor and consultant to uniQure for the development of therapeutic products using his invention of FIX-Padua. He will assist in the Company’s discussions with regulators, investigators, and key opinion leaders throughout the clinical development of AMT-061.

“uniQure is very pleased to have Professor Simioni, a leading expert in hemophilia and FIX-Padua, as a collaborator and to have acquired his patents on the invention of FIX-Padua for gene therapy,” said Jonathan Garen, chief business officer of uniQure. “These patents provide uniQure with an enhanced proprietary position in hemophilia B gene therapy that we can further leverage for additional patent protection.”

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, the development 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, and the scope of protection provided by our patent portfolio. 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, 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 August 8, 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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