

## **uniQure Announces It Will Not Seek Marketing Authorization Renewal for Glybera in Europe**

- Marketing Authorization for Glybera® to Expire on October 25, 2017—
- Company Maintains Focus on Core Programs in Hemophilia B, Huntington's Disease and Congestive Heart Failure --

**Lexington, MA and Amsterdam, the Netherlands**, April 20, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that it will not pursue the renewal of the Glybera® (alipogene tiparvovec) marketing authorization in Europe when it is scheduled to expire on October 25, 2017.

“The decision to not pursue marketing authorization renewal of Glybera in Europe involved a thoughtful and careful evaluation of patient needs and the clinical use of the therapy, and is not related to any risk-benefit concern,” stated Matthew Kapusta, chief executive officer of uniQure. “Glybera’s usage has been extremely limited and we do not envision patient demand increasing materially in the years ahead.”

Mr. Kapusta added, “In line with our previously announced strategy, we will focus our resources on advancing our hemophilia B program into a pivotal trial, moving our Huntington’s disease program into a clinical proof-of-concept trial, and progressing our research and development collaboration with Bristol-Myers Squibb.”

In October 2012, the European Commission granted a five-year marketing authorization for Glybera under exceptional circumstances as a treatment for a small subset of patients with familial lipoprotein lipase deficiency (LPLD), an ultra-rare genetic disorder. As part of Glybera’s approval, uniQure was required to establish a global registry for the long-term surveillance of patients, conduct a post-approval clinical study, submit for annual regulatory reassessments and implement additional risk management procedures. All of these activities required a significant infrastructure for uniQure that included the Company bearing the full costs of maintaining commercial manufacturing capabilities, managing development and validation of numerous assays and supporting regulatory interactions and inspections.

uniQure has initiated discussions with the European Medicines Agency (EMA) to discuss steps to wind down these various activities and review plans for ongoing patient monitoring.

Under the terms of the agreement between uniQure and Chiesi Group, which has exclusive rights for the commercialization of Glybera in Europe and other selected countries, uniQure will continue to make product available to Chiesi to treat any patients that are approved for treatment prior to October 25, 2017, and will also be responsible for terminating the Phase IV post-approval study.

As a result of the withdrawal of Glybera, uniQure expects to reduce future expenses related to the product by approximately \$2 million annually, beginning in 2018 and net of any payments to Chiesi. These cost savings will be in addition to those previously announced by the Company related to the consolidation of manufacturing into the Company’s Lexington facility. uniQure continues to expect its existing cash resources will be sufficient to fund operations into 2019.

[About Glybera:](#)

Glybera is a one time, single-administration gene therapy, which introduces copies of natural LPL gene to produce functional LPL enzyme, providing a long-term therapeutic effect, currently documented up to six years from administration.

Chiesi Group has exclusive rights for the commercialization of Glybera in Europe and other selected countries, as well as for the co-development and commercialization of a gene therapy for hemophilia B.

## **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](http://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 winding down of our Glybera program, the development of our other 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 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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