



## **uniQure Inc. - uniQure Presents New Data Demonstrating Its Large-Scale Manufacturing Capabilities at the European Society of Gene and Cell Therapy (ESGCT) Annual Meeting**

### **~ Commercial Capacity for AAV Gene Therapy Production Significantly Enhanced by Highly Scalable 500 Liter Reactors ~**

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Oct. 17, 2018 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe unmet medical needs, today presented new data demonstrating its ability to manufacture gene therapies using a 500 liter single-use, stirred tank reactor. This presentation was featured at the European Society of Gene and Cell Therapy (ESGCT) 26<sup>th</sup> Annual Meeting in Lausanne, Switzerland.

uniQure produces its adeno associated virus (AAV)-based gene therapies in its state-of-the-art, Lexington-based manufacturing facility using a proprietary baculovirus expression vector system (BEVs). The company recently initiated the development of a 500 liter stirred tank reactor process that has the potential to significantly increase manufacturing capacity and enhance scalability. Analysis of drug substance produced using the 500 liter stirred tank reactor showed that overall process performance, biological activity, content and product purity were comparable to material produced by smaller scale, wave-based reactors.

"AAV is becoming a vector of choice for gene therapy applications, leading to increased demand to advance new therapies for larger patient populations," stated Scott McMillan, Ph.D., uniQure's chief operating officer. "We believe uniQure's proprietary insect cell-based platform provides significant advantages in scaling production for commercial use. Based on our achievements to date, we believe uniQure has the capability to support the manufacturing needs across its expanding pipeline of gene therapy product candidates."

#### **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, the achievement of any of our planned near term or other milestones, our ability to scale production for commercial use, our ability to expand our pipeline, our ability to*

*produce produce or scale gene therapies for commercial use, and/or the development and regulatory approval of our product candidates in the United States or in Europe. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with clinical results, regulatory approval and 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, 2018. 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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