

## uniQure and Gen-X Enter into Research Collaboration and License Agreement for the Development of Novel Synthetic Promoters

~ Gen-X Will Work with uniQure During an Exclusivity Period to Identify Novel Promoters for uniQure's Product Portfolio ~

~ uniQure Receives Option to Acquire Gen-X Business for Use in Gene Therapy ~

**Lexington, MA and Amsterdam, the Netherlands, March 3, 2020** — [uniQure](#) N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, and Gen-X, a spin-off company from the Netherlands Cancer Institute, today announced that they have signed an exclusive research collaboration and license agreement with Gen-X, with an option for uniQure to acquire Gen-X, to identify novel synthetic promoters for use in gene therapy products.

Gen-X performs functional annotation of the non-coding genome using its proprietary Survey of Regulatory Elements (SuRE) technology. This technology measures the potential of hundreds of millions of DNA elements for their ability to serve as promoters or enhancers, driving the gene expression with a range of unique properties for gene therapy applications. The SuRE technology is unique among other promoter-identification technologies in finding non-coding promoter elements throughout the genome at remote sites where activity is regulated.

"Novel promoters are an important area of innovation in gene therapy with the potential to enable valuable functionality and improve treatment options," stated [Jonathan Garen](#), chief business officer of uniQure. "With the unique SuRE methodology developed by Gen-X, we will now leverage our modular technology platform to potentially identify new promoters and enhancers that drive gene expression in the right cell-type and under the right conditions. We are excited about the collaboration, providing us with the potential of applying this state-of-the-art technology and increasing the expression and effectiveness of our next generation gene therapy product candidates."

During the collaboration, Gen-X will work exclusively with uniQure to develop promoters for the uniQure research pipeline. Through the SuRE technology, the Gen-X promoter library is designed to identify novel promoters to regulate gene activity and, if needed, conditionally control protein production. The parties will also explore further development of AAV vector libraries containing fragments of the non-coding human genome. Active promoter elements can be directly identified following administration of these libraries *in vivo*.

"We are very proud to enter into a partnership with uniQure, giving us the opportunity to demonstrate the value of the Gen-X platform in a gene therapy setting," stated Joris van Arensbergen, Gen-X founder and CEO. "We believe that combining our platform with uniQure's leading expertise in AAV gene therapy will accelerate the development of advanced gene therapy candidates."

Until the expiry of the research term of the collaboration, uniQure has an option to acquire the issued and outstanding shares of Gen-X for a pre-agreed price. Financial terms on the research collaboration and license agreement were not disclosed.

### **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 gene therapies to treat patients with hemophilia B, hemophilia A, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other diseases. [www.uniQure.com](http://www.uniQure.com)

### **About Gen-X**

Gen-X is a biotechnology company located in Amsterdam, the Netherlands performing functional annotation of the non-coding genome on an unprecedented scale using its SuRE technology. The company provides screening services to gene therapy companies for the identification of DNA elements that enable controlled transgene expression.

### **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 generation of new technology or products, and the payment of associated future milestones and royalties. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, clinical results, collaboration arrangements, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 2, 2020. 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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