uniQure Announces Development of a Highly Potent, Next-Generation Promoter for Liver-Directed Gene Therapies

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~ New Data Presented at the European Society of Gene and Cell Therapy Annual Meeting Demonstrates up to 40 Times Higher Liver Expression with Optimized Promoter ~

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, Oct. 18, 2018 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today presented non-clinical data demonstrating that a next-generation synthetic promoter developed for liver-directed gene therapy is able to drive gene expression at increased levels and with very high specificity, allowing uniQure to tailor expression levels required for a specific therapeutic transgene. These data were featured in an oral session at the 26th Congress of the European Society of Gene and Cell Therapy (ESGCT), being held from October 16 – 19, in Lausanne, Switzerland.

A "promoter" is an essential component of a gene therapy construct that controls expression of a therapeutic protein. Most gene therapies incorporate natural promoters, which have limitations and may not optimize the expression of genes in specific target cells. Consequently, natural promoters may not be appropriate for gene therapies that require higher levels of gene expression and tissue specificity.

In collaboration with Synpromics Ltd, a U.K.-based developer of gene expression solutions, uniQure has developed synthetic promoters designed to enhance gene expression in a highly specific manner. Two promoter libraries and several data-driven rational designs were analyzed *in-vitro* for strength and specificity. Selected promoters then underwent additional design optimizations and were further validated through non-clinical testing.

Non-Clinical Data Findings:

The first *in-vivo* comparison study was performed in a mouse model injected with AAV incorporating a reporter gene and an optimized promoter candidate. The expression of the reporter gene using the optimized promoter was compared to that using a standard reference promoter. Data from this study showed the optimized promoter was capable of generating up to a 40-fold increase in expression compared to the reference promoter.

A second *in-vivo* comparison study was performed in non-human primates (NHPs) and demonstrated that the optimized promoter was able to express a therapeutically relevant protein in NHPs, maintaining 8-fold higher protein levels over the reference promoter at 8 weeks post-injection.

Additional data from a total of 15 validated promoter designs show that each performed better than the reference promoter in expressing the reporter gene both in *in-vitro* and *in-vivo* studies.

"The data from these studies underscore the potential value of uniQure's gene therapy technology platform built over the last two decades," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "These novel promoters result in a significant increase of the expression of a therapeutically relevant gene, which may prove to be critical to optimizing clinical outcomes for many liver-directed disorders. We believe this proprietary technology will be important in the development of next generation, liver-targeted gene therapy programs, and we look forward to sharing additional details on the applicability of these promoters at our investor-focused R&D Day next month."

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 achievement of any of our planned near term or other milestones, the success of our collaborations, our ability to develop next generation liver-targeted gene therapy programs, 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 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 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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