

uniQure to Acquire Corlieve Therapeutics and Advance its Gene Therapy Program to Treat Temporal Lobe Epilepsy (TLE)

- ~ Expands uniQure's Pipeline of Innovative Gene Therapies to Treat Neurological Disorders ~
- ~ Strengthens uniQure's Global Leadership in the Development of Gene Therapies that Employ miRNA Silencing Technology ~
- ~ Large Opportunity Targeting an Estimated 1.3 million TLE Patients in the U.S. and Europe with Approximately 800,000 Drug-Resistant Patients ~
- ~ Preclinical Proof-of-Concept Data from Disease Model and from Resected Hippocampi from Refractory TLE Patients Demonstrate Clear Suppression of Chronic Spontaneous Epileptic Seizures ~

Lexington, MA; Amsterdam, the Netherlands; and Paris, France, June 22, 2021 — [uniQure N.V.](https://www.uniqure.com) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced it has entered into a definitive agreement to acquire Corlieve Therapeutics and its lead program, which will be known as AMT-260, to treat temporal lobe epilepsy, the most common form of focal epilepsy.

Corlieve's lead gene therapy program employs miRNA silencing technology to target suppression of aberrantly expressed kainate receptors in the hippocampus of patients with temporal lobe epilepsy (TLE). TLE affects approximately 1.3 million people in the U.S. and Europe alone, of which approximately 800,000 patients are unable to adequately control acute seizures with currently approved anti-epileptic therapies. Patients with refractory TLE experience increased morbidity, excess mortality, and poor quality of life.

AMT-260 was originally developed by Corlieve in collaboration with Christophe Mulle, Ph.D., CNRS Research Director, at the Interdisciplinary Institute of Neurosciences, CNRS, University of Bordeaux, Valerie Crepel, Ph.D., Inserm Research Director at the Institut de Neurobiologie de la Méditerranée, INSERM, Aix-Marseille University, and REGENXBIO Inc. Drs. Mulle and Crepel will continue their collaborations with uniQure.

"The acquisition of Corlieve provides an extraordinary opportunity to transform the lives of hundreds of thousands of patients around the world suffering from epilepsy and aligns with our vision of pursuing unmet medical needs for disorders that impact large populations and can be addressed with gene therapies directed to the CNS and liver," stated Matt Kapusta, Chief Executive Officer of uniQure. "The groundbreaking work of the Corlieve team, in collaboration with Drs. Mulle and Crepel and others, has led to compelling preclinical results in temporal lobe epilepsy that we believe can strategically leverage uniQure's leading position in developing and delivering gene therapies that employ miRNA silencing technology. We look forward to welcoming the Corlieve team into the uniQure family as we join forces to advance this important and potentially transformative therapy into clinical studies."

"I am very proud of what Corlieve has achieved in such a short amount of time. With the dedication and focus of our team and our collaboration partners, we have taken a promising therapeutic approach discovered by our

scientific founders and created a potential transformative therapeutic opportunity for patients with refractory TLE,” said Richard Porter, Ph.D., founder and Chief Executive Officer of Corlieve. “As leaders in the field for miRNA gene therapy for neurological conditions, uniQure is the ideal long-term partner for us, and we look forward to working together to advance our program rapidly to the clinic for the benefit of the patients we serve.”

Upon the closing of the transaction, Dr. Porter will assume the role at uniQure of General Manager of the Corlieve subsidiary.

“As a founding investor, we are delighted to see that the translational work conducted by Corlieve is recognized by uniQure, further reinforcing our commitment to company creation based on partnering with the industry,” said Vanessa Malier, Managing Partner at Kurma Partners and Chairman of Corlieve.

Transaction Details

Under the terms of the agreement, uniQure will pay €46.3 million in an upfront payment of cash to acquire Corlieve.

Corlieve shareholders are eligible to receive the following additional payments of which up to 25% will be payable in uniQure ordinary shares at uniQure’s election: up to €43.7 million in development milestones through Phase I/II and €160 million in milestones associated with Phase III development and the approvals of AMT-260 in the U.S and European Union.

Corlieve has an established license and collaboration agreement with REGENXBIO that includes an exclusive license to AAV9 for the specific genetic target of AMT-260. Under the license and collaboration agreement, REGENXBIO received equity in Corlieve and is eligible to receive milestone payments and royalties on net sales of AMT-260.

The transaction has been approved by the Boards of both companies and does not require uniQure shareholder approval. The transaction is subject to customary closing conditions as well as review by the French Ministry of Economy, Finance and Recovery (Ministère de l’Economie, des Finances et de la Relance) pursuant to articles L.151-3 and R.151-1 and seq. of the French Code Monétaire et Financier. Currently, the transaction is anticipated to be completed early in the third quarter of 2021.

SVB Leerink LLC is acting as sole financial advisor and Morgan Lewis is acting as legal advisor to uniQure. McDermott Will & Emery is acting as legal advisor to Corlieve.

About Corlieve Therapeutics

Corlieve Therapeutics is a biotechnology company focused on bringing novel therapeutic options to patients with severe neurological disorders. The lead project is targeting aberrantly expressed kainate receptors in the hippocampus of patients with refractory TLE using a gene therapy approach. Corlieve was founded by Kurma Partners 4Q2019 on the basis of a partnership with REGENXBIO Inc., SATT Aquitaine Science Transfert, and Inserm Transfert. Corlieve is supported by its investors and partners Kurma Partners, Eurazeo, Pureos Bioventures, SATT Aquitaine Science Transfert, Inserm Transfert, Inserm CNRS, and REGENXBIO. For more information, please visit www.corlieve.com.

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, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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, whether uniQure or Corlieve will advance the AMT-260 program to the clinic rapidly or at all, and whether the transaction is completed early in the third quarter of 2021 or ever. uniQure's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our risks associated with the integration of Corlieve and the execution of the AMT-260 development efforts, regulatory review of the transaction, 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 Quarterly Report on Form 10-Q filed on May 10, 2021. 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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