



uniQure Announces Presentations at the Annual Meeting of The European Society of Gene and Cell Therapy (ESGCT)

October 19, 2021

Oral Presentation to Highlight AMT-191 for Fabry Disease

LEXINGTON, Mass. and AMSTERDAM, The Netherlands, Oct. 19, 2021 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that four data presentations, including one oral presentation, will be delivered at the European Society of Gene and Cell Therapy (ESGCT) 28th Annual Meeting taking place virtually today through October 22, 2021.

Featured in the oral presentation are preclinical data of AMT-191, uniQure's gene-therapy candidate for the treatment of Fabry disease which is advancing into IND-enabling studies. AMT-191 is a one-time administered AAV5 gene therapy incorporating an α -galactosidase A (GLA) transgene. New preclinical data confirms high efficiency and cross correction of AMT-191 in a Fabry mouse model, with increased GLA-activity in the liver, kidney, heart, and brain and normalized (lyso-)Gb3 levels in main target organs. In addition, a single administration of AMT-191 led to phenotype improvement of nociception or a faster reaction time to physical stimulus.

These new data build on earlier preclinical studies comparing multiple product candidates, including constructs incorporating a modified alpha-N-acetylgalactosaminidase transgene (modNAGA) where AMT-191 demonstrated robust and sustained increases in GLA activity and subsequent functional improvement. Moreover, pre-clinical studies in non-human primates with AMT-191 demonstrated robust and sustained increases in GLA activity in multiple organs.

"We are enthusiastic about sharing these data on our Fabry program with the scientific community, and we plan to accelerate the preclinical work necessary to file an IND for AMT-191 by 2023," stated [Ricardo Dolmetsch](#), Ph.D., president of research and development at uniQure. "We believe that this preclinical data on AMT-191 showing high efficiency and cross correction in a mouse model differentiates this program from other current investigational gene therapy programs in Fabry disease. We look forward to accelerating this program closer to IND filing and the clinic."

Specific details on uniQure's preclinical data presentations at ESGCT include:

- **Oral Presentation Title:** Pre-clinical proof of concept of an AAV5-GLA gene therapy for Fabry disease results in cross-correction in GLA-KO mice and non-human primates in target organs
Date and Time: Wednesday, October 20, 2021, 4:15 - 4:30 p.m. CEST (10:15 – 10:30 a.m. ET)
Location: Session 3a: Liver & Metabolic Disease Studio 1
Summary: AMT191, the company's investigational AAV5 gene therapy for Fabry disease, containing a proprietary promoter and the GLA enzyme, reduces GB3 accumulation in mouse liver, kidney, heart and brain.
- **Title:** Dose dependent lowering of alpha-synuclein and rescue of motor phenotype by miRNA-based AAV gene therapy
Date and Time: Tuesday, October 19, 2021, 8:00 a.m. CEST (2:00 a.m. ET)
Location: Poster P073
Summary: AMT-230, the company's investigational AAV gene therapy for Parkinson's Disease, reduces alpha synuclein in dopaminergic neurons and rescues motor phenotypes in a genetic model of Parkinson's Disease.
- **Title:** Efficacy of C9orf72 ALS gene therapy using miQURE® and widespread distribution in cortical and spinal regions in non-human primates
Date and Time: Tuesday, October 19, 2021, 8:00 a.m. CEST (2:00 a.m. ET)
Location: Poster P052
Summary: AMT-161, the Company's investigational AAV gene therapy for ALS, reduces the expression of c9ORF72 in human iPSC derived neurons, reduces C9ORF72-containing RNA foci in a model of the disease and is distributed to relevant regions of the spinal cord and brain when expressed in non-human primates.
- **Title:** An AAV9 vector expressing engineered miRNA targeting knockdown of GluK2-containing kainate receptors as a novel gene therapy approach for treating intractable temporal lobe epilepsy
Date and Time: Tuesday, October 19, 2021, 8:00 a.m. CEST (2:00 a.m. ET)
Location: Poster P074
Summary: AMT-260, the Company's investigational AAV gene therapy for temporal lobe epilepsy, efficiently reduces the expression of GluK2 in cortical neurons, reduces epileptiform activity and hyperlocomotion in a preclinical model of epilepsy and blocks epileptiform discharges in organotypic slices from patients with TLE.

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 severe genetic diseases of the central nervous system (CNS) and liver, including clinical programs in hemophilia B and Huntington's disease and preclinical candidates in Fabry disease, spinocerebellar ataxia Type 3, temporal lobe epilepsy, Alzheimer's, Parkinson's, and ALS. www.uniQure.com

unique 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 we will be able to accelerate the preclinical for AMT-191 or file an IND for AMT-191 by early 2023. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the COVID-19 pandemic, our and our collaborators' development activities, collaboration arrangements, strategic shifts, regulatory oversight, 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 July 26, 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.

uniQure Contacts:

FOR INVESTORS:

Maria E. Cantor

Direct: 339-970-7536

m.cantor@uniQure.com

Chiara Russo

Direct: 617-306-9137

c.russo@uniQure.com

FOR MEDIA:

Tom Malone

Direct: 339-970-7558

t.malone@uniQure.com