



uniQure Announces Presentations at the Upcoming Annual Meeting of The American Society of Gene and Cell Therapy (ASGCT)

April 15, 2019

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, April 15, 2019 (GLOBE NEWSWIRE) -- [uniQure N.V.](http://www.uniQure.com) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that six data presentations, of which three are oral presentations, will be delivered at the upcoming American Society of Gene and Cell Therapy (ASGCT) 21st Annual Meeting held in Washington D.C., April 29 – May 2, 2019.

"We have made significant progress over the past year in advancing our gene therapy expertise through expansion of our research pipeline," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "We are very pleased to have such a strong showing at ASGCT, and to have new preclinical data featured in Huntington's disease, hemophilia A and Fabry disease. We look forward to having these data prominently featured at the conference."

Specific details on uniQure's presentations at ASGCT include:

- **Title:** *Sustained Mutant Huntingtin Lowering in the Brain and Cerebrospinal Fluid of Huntington's Disease Minipigs Mediated by AAV5-miHTT Gene Therapy (Abstract #672)*
Oral Session Title: Neurological and Neurosensory Gene Therapy
Date and Time: Wednesday May 1, 4:15 – 4:30 p.m. EDT
Location: Georgetown
- **Title:** *Towards AAV5-mediated Gene Therapy for Hemophilia A with a Factor IX Variant that functions independently of FVIII (Abstract #959)*
Oral Session Title: AAV Vectors and Disease Targets II
Date and Time: Thursday May 2, 11:45 am – 12:00 p.m. EDT
Location: Monroe
- **Title:** *Development of an AAV5-based Gene Therapy for Fabry Disease (Abstract #960)*
Oral session title: AAV Vectors and Disease Targets II
Date and Time: Thursday May 2, 12:00 – 12:15 p.m. EDT
Location: Monroe
- **Title:** *Pre-Existing Anti-Adeno-Associated Virus (AAV) Serotype 5 Neutralizing Antibodies (NABs) Titers in Minipig Serum Do Not Reflect Levels of Anti-AAV5 NABs Titers in Their Cerebrospinal Fluid (CSF) (Abstract #291)*
Poster Session Title: Immunological Aspects of Gene Therapy and Vaccines
Date and Time: Monday Apr 29, 5:00 PM - 6:00 p.m. EDT
Location: Columbia Hall
- **Title:** *Improving AAV Quality Attributes and Process Robustness Through Molecular Redesign (Abstract #343)*
Poster Session Title: Vector and Cell Engineering, Production or Manufacturing
Date and Time: Monday Apr 29, 5:00 PM - 6:00 p.m. EDT
Location: Columbia Hall
- **Title:** *Transfer of Therapeutic miRNAs within Extracellular Vesicles Secreted from Huntington's Disease iPSC-Derived Neurons (Abstract #228)*
Poster Session Title: Neurological Diseases
Session Date and Time: Monday April 29, 5:00 – 6:00 p.m. EDT
Location: Columbia Hall

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 other severe genetic diseases. www.uniQure.com

uniQure Contacts:

FOR INVESTORS:

FOR MEDIA:

Maria E. Cantor

Direct: 339-970-7536

Mobile: 617-680-9452

m.cantor@uniQure.com

Eva M. Mulder

Direct: +31 20 240 6103

Mobile: +31 6 52 33 15 79

e.mulder@uniQure.com

Tom Malone

Direct: 339-970-7558

Mobile:339-223-8541

t.malone@uniQure.com