



uniQure Announces Major Presence at Upcoming American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

May 2, 2023

LEXINGTON, Mass. and AMSTERDAM, May 02, 2023 (GLOBE NEWSWIRE) -- [uniQure N.V.](#) (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that 12 data presentations, including two oral presentations, will be delivered at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting being held May 16-20 in Los Angeles, California.

"Our presentations at ASGCT highlight the innovative work of our uniQure research scientists in expanding our pipeline capabilities, as well as that of our CMC and Operations team with their industry-leading expertise in AAV gene therapy manufacturing," stated [Ricardo Dolmetsch, Ph.D.](#), president of research and development at uniQure. "We are particularly excited to share our progress in developing a gene therapy approach to treating a form of ALS caused by mutant C9orf72."

Specific details on uniQure's presentations taking place at ASGCT include:

- **Title:** *AAV vector DNA carrying two concatenated miRNA stem-loops is highly homogenous and stable in GLP-grade AAV product* (Abstract #110)
Presenter: Chenxia He, Director, Gene Therapy Technology Innovation, uniQure
Oral Session Title: AAV Vectors - Product Development Manufacturing: Analytics & Stability Studies
Date and Time: Thursday, May 18, 1:45 – 2:00 p.m. PDT
- **Title:** *Lowering of toxic dipeptide proteins and phenotype rescue in an ALS mouse model treated with AAV-miQURE® targeting the repeat expansion-containing C9orf72 transcripts* (Abstract #165)
Presenter: Vanessa Zancanella, Sr. Scientist Global Research, uniQure
Oral Session Title: Neurological Gene Therapies in Advanced Stages of Clinical Translation
Date and Time: Thursday, May 18, 3:45 – 4:00 p.m. PDT
- **Title:** *The capsid race: multiplexed analysis and comparison of rationally engineered AAV CNS capsids in vivo* (Abstract #448)
Presenter: Leonard Khirug, Senior Director, Technology Innovation, uniQure
Poster Session Date and Time: Wednesday, May 17, 12:00 p.m. PDT
- **Title:** *Phase-appropriate viral clearance studies for AAV manufacturing process* (Abstract #415)
Presenter: Aidan Hawkins, Engineer III DSP, Drug Substance Development, uniQure
Poster Session Date and Time: Wednesday, May 17, 12:00 p.m. PDT
- **Title:** *Next-generation Rep-Cap Dual baculoviruses for AAV production in insect cells* (Abstract #422)
Presenter: Finny Varghese, Sr. Scientist Virology, Baculovirus Production Unit, Biological Starting Materials, uniQure
Poster Session Date and Time: Wednesday, May 17, 12:00 p.m. PDT
- **Title:** *Development of a gene therapy for Alzheimer Disease by lowering toxic APOE while simultaneously overexpressing a protective APOE variant* (Abstract #373)
Presenter: Amila Zuko, Scientist, Global Research, uniQure
Poster Session Date and Time: Wednesday, May 17, 12:00 p.m. PDT
- **Title:** *Preclinical proof-of-concept of AMT-260, a novel AAV9-dual microRNA-based vector targeting GRIK2 for the treatment of temporal lobe epilepsy* (Abstract #1159)
Presenter: Nick Pearson, Sr. Director Toxicology & Translational Safety, Non-Clinical, uniQure
Poster Session Date and Time: Thursday, May 18, 12:00 p.m. PDT
- **Title:** *Novel method of producing AAV capsid libraries in insect cells* (Abstract #1230)
Presenter: Leonard Khirug, Senior Director, Technology Innovation, uniQure
Poster Session Date and Time: Thursday, May 18, 12:00 p.m. PDT
- **Title:** *Insect cell baculovirus expression platform for large scale production of rAAV* (Abstract #1014)
Presenter: Sandra Bennun, Associate Director/Team Lead USP, Drug Substance Development, uniQure
Poster Session Date and Time: Thursday, May 18, 12:00 p.m. PDT

- **Title:** *Heat Stress on Baculovirus Infected SF+ Cells May Lead to Product Loss During Harvest* (Abstract #910)
Presenter: Robin Mailhot, Sr. Engineer II USP, Drug Substance Development, uniQure
Poster Session Date and Time: Thursday, May 18, 12:00 p.m. PDT
- **Title:** *Development of AbQURE™, an a-synuclein targeting vectorized antibody strategy: First in vivo proof of mechanism in wild type mice* (Abstract #1026)
Presenter: Şeyda Açar Broekmans, Sr. Scientist, Global Research, uniQure
Poster Session Date and Time: Thursday, May 18, 12:00 p.m. PDT
- **Title:** *Combining vectorized antibody and miRNA lowering strategies for synucleinopathies* (Abstract #1503)
Presenter: Şeyda Açar Broekmans, Sr. Scientist, Global Research, uniQure
Poster Session Date and Time: Friday, May 19, 12:00 p.m. PDT

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The recent approvals of our gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represent a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology platform to advance a [pipeline](#) of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, ALS, Fabry disease, and other severe diseases. www.uniQure.com

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