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

April 28, 2020

~ 22 Presentations at ASGCT, Including New Preclinical Data on Gene Therapy Candidates, Highlight uniQure's Industry-Leading Research and Technology Capabilities ~

LEXINGTON, Mass. and AMSTERDAM, April 28, 2020 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that 22 data presentations, of which five are oral presentations, will be delivered at the American Society of Gene and Cell Therapy (ASGCT) Virtual 2020 Annual Meeting being held May 12-15.

"uniQure's significant scientific presence at this year's ASGCT Annual Meeting demonstrates the robustness of our research capabilities and our excellence in developing novel technologies and commercial-scale gene therapy manufacturing," stated Sander van Deventer, executive vice president of research and product development at uniQure. "We are very pleased to present new preclinical data on our gene therapy candidates for hemophilia A, spinocerebellar ataxia type 3, Fabry disease and data on our advances in technology and manufacturing."

Specific details on uniQure's virtual oral presentations at ASGCT include :

Title: *One-Time Intrathecal Administration of AAV5-miATXN3 in Non-Human Primates*
Date and Time: Wednesday May 13, 4:15 p.m. EDT/ 10:15 p.m. CET

Title: *A Novel NAGA Variant Designed to be Non-immunogenic In Humans and Provide Broad Cross-Correction in Fabry Disease*
Date and Time: Thursday May 14, 4:15 p.m. EDT/ 10:15 p.m. CET

Title: *A Single Administration of AAV5-hFIX in Newborn, Juvenile and Adult Mice Leads to Stable hFIX Expression up to 18 Months after Dosing*
Date and Time: Thursday May 14, 4:15 p.m. EDT/ 10:15 p.m. CET

Title: *Characterizing Next-Generation Baculovirus Transduction Processes - A Quality by Design-based Approach for AAV Manufacturing*
Date and Time: Friday May 15, 10:45 a.m. EDT/ 4:45 p.m. CET

Title: *Clearance of Vector DNA From Bodily Fluids in Patients with Severe or Moderate-Severe Hemophilia B Following Systemic Administration of AAV5-hFIX and AAV5-hFIX Padua*
Date and Time: Friday May 15, 11:15 a.m. EDT/ 5:15 p.m. CET

The following presentations were approved for poster presentation:

- *Lipid Nanoparticle Pre-Treatment Improves rAAV Diffusion in the Primate Liver and Enables an Increase of Therapeutic Transgene Expression*
- *Prevalence and Avidity Assessment of Pre-existing Neutralizing Antibodies (NABs) Against Adeno-Associated Virus (AAV) Vector Serotypes 2, 5 And 8 Analyzed in The Serum Of 300 Healthy Donors*
- *AAV Biology Imaging Platform: Uncovering the Cellular Mechanisms Behind the AAV5-Based Vector Delivery System*
- *Development and Optimization of a qPCR Method to Assess Biodistribution of an AAV5 Vector In NHP And Mouse Studies*
- *Translatable Biomarkers in Gene Therapy for Huntington Disease: Innovative Approaches and Learnings from Pre-Clinic to the Clinic*
- *Exploring the Effects of Intrastriatal AAV5-miHTT Lowering Therapy on Transcriptional Dysregulation, MRS Signal, and Mutant Huntingtin Levels in the Q175FDN Mouse Model of Huntington's Disease*
- *Secreted Therapeutics: Monitoring Durability of microRNA-based Gene Therapies in Huntington's Disease*
- *Lowering the Pathogenic Exon 1 HTT Fragment by AAV5-miRNA Gene Therapy*
- *Human Dose Prediction of a Novel Factor IX Variant Gene Therapy Candidate (AMT-180) Mediating Clotting Independently of Factor VIII*
- *Assessment of miQURE™ Efficacy and Safety in SCA3 Neurons*
- *Assessment of the Novel AAV-Based miQURE™ Gene Therapy in SCA3 Animal Models*
- *Development of an AAV5-Based Gene Therapy for Dyslipidemia*
- *Generation and Optimization of Insect Based Stable AAV Production Cell-Line*
- *Generation of a DuoBac Expression System for Robust and High Quality AAV Production*

- *From Rocking Motion Bioreactors to Stirred Tank Bioreactors the Journey to Scalable AAV Gene Therapy*
- *Production of Recombinant AAV Vectors in Chemically Defined Media*
- *Overcoming Challenges for Developing AAV Purification Process for Large-Scale GMP Manufacturing*

The abstracts were published today at the ASGCT Annual Meeting website.

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, hemophilia A, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other diseases. www.uniQure.com

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