uniQure

uniQure initiates Phase I in acute intermittent porphyria
AIPGENE consortium sets first step towards cure for rare disease

Amsterdam, The Netherlands – December 11, 2012 – uniQure B.V., a leader in the field of human gene therapy, today announced the start of its Phase I clinical trial in acute intermittent porphyria (AIP) with the treatment of the first patient. The study is conducted under the aegis of the AIPGENE consortium, a pan-European collaboration funded in part by the European Commission's Seventh Framework Program with the aim to develop a gene therapy for the treatment of AIP, a rare and devastating disease caused by mutations in the porphobilinogen deaminase gene (PBGD). AIP can be life-threatening and the long-term effects include irreversible nerve damage, liver cancer and kidney failure. uniQure was granted orphan drug designation for the treatment of AIP in 2009 from the European Medicines Agency.

"The start of the AIP Phase I study marks the first of four programs that will enter clinical trials over the next 12 months," says Jörn Aldag, CEO of uniQure. "After AIP we expect clinical trials to be initiated in Parkinson's disease, hemophilia B, and Sanfilippo B. After many years of building and developing our capabilities and competencies, and the approval in November of Glybera for LPLD as the first gene therapy in the Western world, we are highly motivated to expedite the clinical development of our other advanced gene therapies."

About the AIP Phase I study
The Phase I will enroll eight patients with severe AIP at two centers: the Clinical University of Navarra, Pamplona, Spain, and the 12 de Octubre University Hospital,, Madrid, Spain. The study's primary objective is the assessment of safety and determination of the maximum tolerated dose. Secondary objectives include tolerability of treatment, pharmacokinetics, changes in the levels of surrogate markers of activity including porphobilinogen (PBG) and delta-aminolevulinic acid (ALA), and assessment of symptom control, neuro-psychological changes and quality of life. All patients will be followed for one year, and the interim results of the Phase I are expected in Q3 2013.

About acute intermittent porphyria
Acute Intermittent porphyria (AIP) is a rare genetic disease which is caused by mutations in the porphobilinogen deaminase (PBGD) gene; one of the enzymes of the heme biosynthesis pathway. Mutations in this gene cause insufficient activity of the protein resulting in partially disruption of heme synthesis. This in turn leads to accumulation of toxic intermediates (ALA and PBG) giving rise to a wide variety of problems including acute, severe abdominal pains, psychiatric and neurological disorders, and muscular weakness. Acute porphyric attacks can be life-threatening and the long-term consequences include irreversible nerve damage, liver cancer and kidney failure. Currently, the only curative therapy is liver transplantation and thus, new curative options are urgently needed. Severe AIP patients are suffering poor quality of life with palliative treatments for the different symptoms including glucose or heme infusions for metabolic replacement and inhibition of toxic metabolic production.

About AIPGENE
AIPGENE is a European Commission Framework Programme 7-funded consortium (Grant Agreement number 261506) which was put together with the aim to develop the orphan gene therapy drug AAV5-AAT-PBGD (AMT-021) for the treatment of Acute Intermittent porphyria (AIP). The consortium's objective is to contribute to alleviating the negative impact of this disease on the quality of life of the patients and their families. Overall coordinator of the project is the Centre for Applied Medical Research (CIMA) at the University of Navarra, Pamplona, Spain. Apart from uniQure, other members of the consortium are the Clinical University of Navarra, Pamplona, Spain; Karolinska University Hospital, Stockholm, Sweden; German Cancer Research Center (NCT-DKFZ), Heidelberg, Germany; DIGNA Biotech, Pamplona, Spain; Servicio Madrileno de Salud, Madrid, Spain.

About uniQure
uniQure is a world leader in the development of human gene based therapies. uniQure's Glybera, a gene therapy for the treatment of lipoprotein lipase deficiency has been approved in the European Union, and is the first approved gene therapy in the Western world. uniQure's product pipeline of gene therapy products in development comprise hemophilia B, acute intermittent porphyria, Parkinson's disease and Sanfilippo B. Using adeno-associated viral

(AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate probably the world's first stable and scalable AAV manufacturing platform. This proprietary platform can be applied to a large number of rare (orphan) diseases caused by one faulty gene, and allows uniQure to pursue its strategy of focusing on this sector of the industry. uniQure's largest shareholders are Forbion Capital Partners and Gilde Healthcare, two of the leading life sciences venture capital firms in the Netherlands. Further information can be found at www.uniqure.com.

For further enquiries:

Jörn Aldag
CEO
uniQure
Tel : +31 20 566 8014
j.aldag@uniQure.com

Hans Herklots
Managing Director
Capricorn One
+41 79 598 7149
capricorn1@bluewin.ch