uniQure

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uniQure Announces Successful Transfection of Liver Cells with AAV5 and Strong Safety Data from Acute Intermittent Porphyria Clinical Trial

-Phase I Interim Results of AMT-021 Presented at ASGCT Conference--

Amsterdam, the Netherlands, May 27, 2014 — uniQure N.V. (Nasdaq: QURE), a leader in human gene therapy, today announced successful transfection of liver cells with the porphobilinogen deaminase gene (PBGD) from an ongoing Acute Intermittent Porphyria (AIP) dose-escalation Phase I trial conducted in collaboration with the AIPGENE Consortium, a pan-European collaboration. The results, presented Saturday, May 24, at the 17th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) by Dr. Gloria González-Aseguinolaza from the AIPGENE Consortium also provided evidence that the AAV5 vector from uniQure's bacculovirus production platform is safe by inducing liver cell transfection in AIP patients without liver enzyme perturbations at the tested dose levels.

AIP is a rare and devastating monogenic disease caused by mutations in the PBGD gene. AIP can be life threatening and the long-term effects include irreversible nerve damage, liver cancer and kidney failure. The AAV5-PBGD gene therapy candidate (AMT-021) consists of the PBGD gene encapsulated in uniQure's proprietary AAV5 viral vector and is designed to enable transfection of the PBGD gene into patient's liver cells through a single therapeutic intervention.

In the ongoing multicenter, open label, single dose, dose-ranging Phase I clinical trial, a total of eight patients in four dosing cohorts received AMT-021 doses of up to 2 x 10^{13} gc/kg (see table below). The trial reported no safety concerns from any patient based on review at six months post administration. In particular, no liver enzyme perturbations have been noted at any dose level and no administration of corticosteroids has been required. The result of liver biopsies performed between week 42 - 47 in six out of the eight patients show the presence of PBGD DNA in the liver parenchymal cells demonstrating that uniQure's exclusively licensed AAV5 vectors can be effective in infecting human liver cells and delivering the PBGD gene into liver cells.

Vector		**Documented AAV transfection**		**S-ALT levels**		
Serotype	*Dose (gc/kg)*	*Liver biopsy*	*Detectable PBGD DNA*	*Peak (IU/L)*	*Time*	*Immune Suppression*
AAV5	5 x 10^{11}	Yes	+	WNL	6 months	No
AAV5	5 x 10^{11}	Yes	++	WNL	6 months	No
AAV5	2 x 10^{12}	Yes	+	WNL	6 months	No
AAV5	2 x 10^{12}	Yes	++	WNL	6 months	No
AAV5	6 x 10^{12}	No	N/A	WNL	6 months	No
AAV5	6 x 10^{12}	Yes	+++	WNL	6 months	No
AAV5	2 x 10^{13}	Yes	++	WNL	6 months	No
AAV5	2 x 10^{13}	No	N/A	WNL	6 months	No
Abbreviations: WNL, within normal limits; AAV, adeno-associated virus; ALT, alanine aminotransferase						

"The transfection of liver cells with evidence of transgene expression and with no safety concerns to date is the first documented evidence of the potential clinical utility of our AAV5 vector for liver-directed gene therapy. AAV5 is an important part of our gene therapy platform and the vector used in our hemophilia B and Sanfilippo B gene therapies," said Dr. Harald Petry, Chief Scientific Officer of uniQure. "The Consortium will release full data from the clinical study later in 2014. Based on those results, uniQure will define the next steps for further clinical evaluation of AMT-021."

AMT-021 aims to provide long-term normalization of the PBGD protein in order to prevent acute AIP attacks and the associated complications. The ongoing Phase I trial, conducted by Digna Biotech as part of the AIPGENE Consortium, has enrolled eight patients with severe AIP. The study's primary objective is to assess the safety of systemic administration and to determine the maximum tolerated dose of AMT-021. Secondary objectives include exploring urinary levels of toxic metabolites to determine their adequacy as biomarkers of treatment effect. uniQure received orphan drug designation for AMT-021 in 2009 from the European Medicines Agency.

About AIP

Acute Intermittent Porphyria (AIP) is a rare metabolic liver disorder resulting from mutations in the porphobilinogen deaminase, or PBGD, gene, which encodes for the enzyme involved in the production of heme in the body. Insufficient activity of this protein leads to an accumulation of toxic metabolites, resulting in a wide variety of serious clinical problems, including acute, severe abdominal pain, muscular weakness and an array of neurologic manifestations, including psychiatric episodes, seizures and coma. In the majority of cases, precipitating factors, such as hormonal fluctuations, infections, drugs and dietary changes, trigger attacks. Long-term consequences may include irreversible nerve damage, liver cancer and kidney failure. Patients with AIP experience regular hospitalizations and extremely poor quality of life. Acute attacks can be life threatening. Current therapies include intravenous administration of heme and carbohydrate loading, which aim to treat the symptoms only and do not prevent attacks. In some cases, AIP patients require liver transplants.

About uniQure

uniQure is delivering on the promise of gene therapy through single treatments with potentially curative results. We have developed a modular platform to rapidly bring new disease-modifying therapies to patients with severe disorders. We are engaged in multiple partnerships and have obtained regulatory approval of our lead product, Glybera, in the European Union for a subset of patients with LPLD. www.uniQure.com

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