

FOR IMMEDIATE RELEASE

Positive Topline Results Announced from Phase I/II Trial in Sanfilippo B Syndrome Patients Using uniQure's Novel AAV5-Based Gene Therapy

- Safety, Durable NaGlu Protein Expression and Positive Signs of Efficacy Demonstrated in All Four Patients--
- First Clinical Validation of AAV5 Vector Effectively Delivering Target Gene into the CNS--
- Company Intends to Advance Program into Pivotal Stage--
- Conference Call to Discuss Data Scheduled for 8:30 am EDT Monday, September 21--

Amsterdam, the Netherlands, September 19, 2015 —uniQure N.V. (Nasdaq: QURE), a leader in human gene therapy, today announced the topline results of one-year follow-up data from a Phase I/II clinical trial conducted by Institut Pasteur (Biotherapies for Neurodegenerative Diseases Unit, Institut Pasteur/INSERM) in partnership with the French Muscular Dystrophy Association and Vaincre les Maladies Lysosomales (collectively “the consortium”) in four Sanfilippo B syndrome (MPSIIIB) patients treated with a novel gene therapy, AMT-110. In all four patients, researchers verified the restoration of catalytic activity of the NaGlu protein in the cerebrospinal fluid (CSF) from 0% at baseline up to 14-17% of normal at 3 months with persistent effect at 12 months. These results validate the effective transmission of the NAGLU gene with the AAV5 vector. The trial demonstrated that incremental cognitive development was maintained in all four patients, aged 20 to 53 months at study onset. The therapy consists of uniQure's proprietary AAV5 viral vector and a gene cassette including the N-acetylglucosaminidase, alpha (NAGLU) gene, manufactured with uniQure's proprietary insect cell based-technology. The results of the trial were presented on September 19th at the European Society of Gene and Cell Therapy (ESGCT) and Finnish Society of Gene Therapy (FSGT) Collaborative Congress held in Helsinki, Finland, by Dr. Marc Tardieu, Professor at the Université and Hôpitaux Universitaires Paris Sud and primary investigator of the trial with co-investigators Professors Jean-Michel Heard and Michel Zerah.

“We are forging new ground in the treatment of Sanfilippo B with this study. For the first time we have shown persistent restoration of NaGlu protein expression over 12 months can be achieved with gene therapy in the CNS,” said Professor Tardieu. “Published case studies of attenuated Sanfilippo B patients support that NaGlu protein activity in the range of 5-10% of normal can be considered clinically meaningful and is associated with longer lifespans and higher quality of life for Sanfilippo B patients. We look forward to further confirming these encouraging trends on cognitive development over the next 6-12 months.”

In the trial, four patients received a one-time administration of AAV5 gene therapy dosed over two hours directly into the brain. All patients were maintained under coverage of a continuous immune suppression regimen. No local inflammation or other safety concerns related to the therapy or the procedure have been identified. In addition to establishing the safety of the procedure and the AAV5 viral vector, the most important result was the presence of catalytically active NaGlu protein in the CSF measured at 1, 3, and 12 months after treatment. The fact that all treated Sanfilippo B subjects continued to gain skills throughout the study is extremely encouraging.

“We are gratified that our pursuit of a gene therapy targeting CNS diseases has found this early and promising success. The data validates AAV5 from our insect cell-based manufacturing platform as a safe and effective vector choice for CNS administration. Based on these results, we are negotiating an agreement with the consortium to take over the sponsorship of the program and we intend to advance the program into the pivotal stage of development. We acknowledge the tremendous support we have received from the consortium that was paramount for generating this early proof in patients,” said Joern Aldag, CEO of uniQure. “Sanfilippo B syndrome represents a high unmet medical need and we are committed to developing a gene therapy that can improve and extend the lives of patients and making it available to them and their families worldwide as soon as possible.”

About Sanfilippo B

Sanfilippo B is a rare inherited lysosomal storage disease that results in serious brain degeneration in children and is generally fatal. The disease stems from an enzyme deficiency caused by a malfunctioning NAGLU gene. Due to the inability to properly break down long chains of sugar molecules called mucopolysaccharides or glycosaminoglycans (GAGs) that are a normal by-product of cell metabolism, the cells accumulate partially degraded oligosaccharides of heparin sulfate, which are molecules that regulate various developmental processes. The clinical manifestations of the disease are mainly neurological with early symptoms observed during the first 5 years of age, leading to a progressive deterioration of cognitive abilities. Affected children begin developing cognitive symptoms at around 2 years of age, proceeding to a precipitous neurological decline with behavioral, sleep and social difficulties between the ages of 4 and 7. Further profound mental retardation ensues, with death occurring around age 17. No proven disease modifying treatments for Sanfilippo B are currently available.

Conference Call and Webcast

uniQure will discuss the data in a webcast conference call at 8:30 am EDT on Monday, September 21, 2015. The conference call can be accessed by dialling one of the numbers listed below five minutes prior to the start of the call and providing the confirmation code: **3342119**.

Local - London, United Kingdom:	+44(0)20 3140 8286
Local - New York, United States of America:	+1 212 444 0412
Local - Berlin, Germany:	+49(0)30 3001 90539
Local - Amsterdam, Netherlands:	+31(0)20 716 8296
Local - Milan, Italy:	+3902 3600 9869
Local - Paris, France:	+33(0)1 76 77 22 28
Local - Brussels, Belgium:	+32(0)2 402 3092
Local - Montreal, Canada:	+1514 841 2153

Investors may also listen to the webcast of the conference call live on the "Events" section of uniQure's website, www.uniQure.com. To ensure a timely connection to the webcast, it is recommended that users register at least 15 minutes prior to the scheduled start time. The webcast replay will be available for at least 72 hours following the call.

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 CNS, liver/metabolic and cardiovascular diseases. www.uniQure.com

FORWARD-LOOKING STATEMENTS

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, statements regarding the progress or timing of the further development of AMT-110, the potential long-term clinical benefit of AMT-110, the ability to demonstrate the efficacy of AMT-110 in larger, late-stage clinical trials, and our ability ultimately to bring AMT-110 to market. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our clinical development activities, manufacturing processes and facilities regulatory oversight, product commercialization, intellectual property claims, risks associated with our collaborations and collaboration partners, and the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's Form 20-F filed with the Securities and Exchange Commission dated April 7, 2015. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future. www.uniQure.com

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