For immediate release
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Forbion Portfolio Company uniQure receives Approval for Glybera® First Gene Therapy by European Commission

- **Glybera becomes the first gene therapy approved by regulatory authorities in the Western world**
- **First medication approved for patients with rare metabolic disorder Lipoprotein Lipase Deficiency**
- **Commercial roll-out to begin second half of-2013**
- **Validates uniQure's unique AAV-based gene therapy platform**

Naarden, The Netherlands, – November 2, 2012 – Forbion Capital Partners is pleased to announce that its investee company, uniQure received approval today from the European Commission (EC) for the gene therapy Glybera® (alipogene tiparvovec), a treatment for patients with lipoprotein lipase deficiency (LPLD, also called familial hyperchylomicronemia) suffering from recurring acute pancreatitis. Patients with LPLD, a very rare, inherited disease, are unable to metabolize the fat particles carried in their blood, which leads to inflammation of the pancreas (pancreatitis), an extremely serious, painful, and potentially lethal condition. The approval makes Glybera the first gene therapy approved by regulatory authorities in the Western world.

"Glybera's approval means LPLD patients, for the first time, have a medical treatment option for a very complex and severe disease," said Professor John Kastelein of the Department of Vascular Medicine at the Academic Medical Center of the University of Amsterdam, the Netherlands. "LPLD leads to acute and recurrent pancreatitis attacks, and in many patients causes early onset diabetes and cardiovascular complications. This therapy will have a dramatic impact on the lives of these patients. Currently their only recourse is to severely restrict the amount of fat they consume. By helping to normalize the metabolism of fat, Glybera prevents inflammation of the pancreas thereby averting the associated pain and suffering and, if administered early enough, the associated co-morbidities."

As part of the approval, patients will receive treatment with Glybera through dedicated centers of excellence and by specially trained doctors. uniQure will also build a patient registry to further improve the understanding of this devastating, under-researched disease and the effects of Glybera treatment. Marketing Authorisation covers all 27 European Union member states. uniQure is preparing to apply for regulatory approval in the US, Canada, and other markets.

"The final approval of Glybera from the EC marks a major step forward in making gene therapies available not only for LPLD but also for a large number of rare diseases with a very high unmet medical need," says Jörn Aldag, CEO of uniQure. "The EC's approval is an important validation of our innovative product platform and offers strong support for our other advanced development programs, which focus on acute intermittent porphyria, Sanfilippo B, hemophilia B and Parkinson's disease."

"Forbion has supported the company from the first VC round through to today's marketing approval of its gene therapy product, Glybera. The experience has been very rewarding, not

only to us as an investor, but most importantly in terms of providing a new treatment to patients suffering from this rare and debilitating disease," said Sander Slootweg, Managing Partner at Forbion Capital Partners.

Sander van Deventer, General Partner at Forbion Capital Partners added: "This European Medicines Agency approval has paved the way for additional uniQure products to follow by using its modular, plug-and-play gene therapy platform. Porphyria, another serious and rare disease, is being targeted next. Given the number of diseases amenable to gene therapy, we believe there will be many partnering opportunities open to uniQure in the future."

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About Forbion Capital Partners

Forbion Capital Partners is a dedicated Life Sciences venture capital firm with offices in Naarden, The Netherlands, and Munich, Germany. Forbion invests in life sciences companies in drug discovery & development as well as medical device companies addressing substantial unmet medical needs. Forbion's investment team of nine investment professionals has built an impressive performance track record since the late nineties with successful investments in Rhein Biotech, Crucell, Neutec, Glycart, Borean, Impella, Alantos, Acorda, Fovea, PanGenetics, Argenta Discovery and most recently Biovex and Pathway Medical. Current assets under management exceed $500M, split between three active funds and comprising some 28 promising portfolio companies. Forbion Capital Partners Fund II is supported by the European Investment Fund through its ERP and LfA facilities. Forbion co-manages Biogeneration Ventures, an early stage fund focused on (academic) spin-outs and seed investments in the Netherlands. For more information, please visit www.forbion.com.

About Glybera®

uniQure has developed Glybera as a therapy for patients with the genetic disorder lipoprotein lipase deficiency, an orphan disease for which no treatment existed. The disease is caused by mutations in the LPL gene, resulting in highly decreased or absent activity of LPL enzyme in patients. This enzyme is needed in order to break down large fat-carrying particles that circulate in the blood after each meal. When such particles, called chylomicrons, accumulate in the blood, they may obstruct small blood vessels. Excess chylomicrons result in recurrent and severe acute inflammation of the pancreas, called pancreatitis, the most debilitating complication of LPLD. Glybera has orphan drug designation in the EU and US. LPL Deficiency affects 1-2 persons per million.

Glybera has been tested in three interventional clinical studies conducted in the Netherlands and in Canada, in which a total of 27 LPLD patients participated. In all three clinical trials, Glybera was well tolerated, with no relevant safety issues observed. Data from these clinical trials indicate that a single dose administration of Glybera resulted in a long-term biological activity of the LPL protein. For further information on LPLD visit www.lpldeficiency.com.

Lipoprotein lipase is a key 'first step' enzyme in the metabolism of lipoproteins following fat intake with diet. In clinical studies a transient reduction in triglycerides for up to 12 weeks in individual patients could be observed. Furthermore, Glybera allows expression of the LPL protein in injected muscle which is reflected by the improvement of postprandial chylomicron (CM) metabolism observed in a small subset of patients.Glybera (Alipogene tiparvovec) contains the human lipoprotein lipase (LPL) gene variant LPL^{S447X} in a vector. The vector comprises a protein shell derived from adeno-associated virus serotype 1 (AAV1), the promoter, a posttranscriptional regulatory element and AAV2 derived inverted terminal repeats.

Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions**.** The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein.

The most commonly reported adverse reaction is pain in extremity occurring in approximately one third of patients. Given the small patient population and size of the cohorts, observed adverse reactions do not provide a complete perspective on the nature and frequency of these events.

About uniQure

uniQure is a world leader in the development of human gene based therapies. uniQure product pipeline of gene therapy products in development comprise hemophilia B, acute intermittent porphyria, Parkinson's disease and SanfilippoB. 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.