**For immediate release**
**20 July 2012**

# Forbion portfolio company, uniQure receives positive opinion for Glybera® in Europe from CHMP

## First gene therapy in Western world recommended for approval

- **First gene therapy in the Western world to reach important regulatory approval milestone, culminating 40 years of research**
- **First therapy for LPL deficient patients, a severe disease with no alternative treatment**
- **Validates uniQure's unique AAV-based gene therapy platform, consisting of a modular, plug-and-play vector system and unrivaled GMP manufacturing capabilities on a commercial scale**
- **Heralds new phase in uniQure's development, including potential revenues from sales and partnerships**
- **Technology platform can now be leveraged to find solutions for many more severe genetic and other disorders**

**Naarden, The Netherlands, 20 July 2012** - Forbion Capital Partners is pleased to announce, that its investee company uniQure has received a positive opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) that recommends marketing authorization of Glybera® (alipogene tiparvovec) as a treatment for lipoprotein lipase deficiency (LPLD) under exceptional circumstances. LPLD is a very rare, inherited disease. Patients with the disease are unable to handle fat particles in their blood plasma, which leads to recurring severe abdominal pain and pancreatitis.

The European Commission (EC) generally follows the recommendations of the CHMP. The Company expects final approval from the EC within 3 months after the CHMP decision. After today's positive recommendation, Glybera is poised to become the first in a class of gene therapy products approved in Europe to treat orphan diseases, rare conditions with a very high unmet medical need. Marketing authorization covers all 27 European Union member states.

Mr. Aldag, CEO of UniQure said: "Patients with LPLD are afraid of eating a normal meal because it can lead to acute and extremely painful inflammation of the pancreas, often resulting in a visit to intensive care. Now, for the first time, a treatment exists for these patients that not only reduces this risk of getting severely sick, but also has a multi-year beneficial effect after just a single injection. The positive recommendation from the CHMP for Glybera therefore represents a major breakthrough for both LPLD patients and for medicine as a whole. Restoring the body's natural ability to break down fat particles in the blood in order to prevent pancreatitis and excruciating abdominal pain suffered by patients, is what gene therapy is all about: curing disease at the genetic level."

"Over the years, we have been closely involved with uniQure, formerly AMT, as financial backers, but also, driving its strategy and specifically, leading the initiative to take AMT private earlier this year to form uniQure," said Sander van Deventer, General Partner at Forbion. We have always been firm believers that after antibodies and lysosomal storage disease enzymes,

gene therapies would constitute the next major class of biotech drugs to reach the markets and become of interest to big pharma. This was proved right in our backing of BioVex which had a gene therapy element to the mode of action of its lead drug OncoVex and was acquired by Amgen for up to $1bn last year. As there are approximately 6,000 mono-genetic diseases amenable to some form of gene therapy, we believe the sky is now the limit for uniQure."

As part of the approval, treatment with Glybera will be offered through dedicated centers of excellence with expertise in treating LPLD and by specially trained doctors to ensure ongoing safety of this novel treatment method. uniQure has also committed to building a patient registry for continued understanding of this devastating, under-researched disease. The Company is now preparing to apply for regulatory approval in the US, Canada, and other markets.

Sander Slootweg, Managing Partner at Forbion, added, "Today's decision to recommend for approval the first gene therapy is a great result after a long journey for both uniQure and for gene therapy as a whole. With Glybera approved in Europe, hopefully other markets will follow soon, so that patients all over the world can benefit from the treatment for the debilitating disease of LPLD. With this approval, uniQure has clearly established itself as the leader in the field of AAV mediated gene therapies. The Company has a rich pipeline of other programmes soon to be tested in the clinic, including a hemophilia B product, so we expect uniQure to become a significant contributor to the Forbion funds' returns."

The uniQure team is also developing treatments for a number of other rare diseases, such as acute intermittent porphyria and Sanfilippo B. The potential of gene therapy stretches far beyond rare diseases. As shown recently in a publication in the New England Journal of Medicine (N Engl J Med 2011; 365:2357-2365, December 22, 2011), hemophilia patients treated with uniQure's proprietary gene are showing a sustained clinical effect over several years, which has allowed prophylaxis treatment to be stopped. In addition, the Company is advancing programs in degenerative diseases such as Parkinson's.

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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 uniQure**
uniQure is a world leader in the development of human gene based therapies. uniQure has a product pipeline of gene therapy products in development for 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 Heatlhcare, two of the leading life sciences venture capital firms in the Netherlands. Further information can be found at www.uniqure.com.

**About Glybera®**
uniQure has developed Glybera as a therapy for patients with the genetic disorder lipo protein lipase deficiency. LPLD is an orphan disease for which no treatment exists today. The disease is caused by mutations in the LPL gene, resulting in highly decreased or absent activity of LPL protein in patients. This protein 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. For further information on LPLD visit www.lpldeficiency.com.

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.