Research Note

uniQure

Gene Therapy programs fairly valued

uniQure

New York, 13 April 2015

Name:	**uniQure**
Country:	**The Netherlands**
Price:	**USD 28.44**
ISIN Code:	**NL0000886968**
Reuters Code:	**QURE (NASDAQ)**
Market Cap (USD m):	**524.0**
EV (USD m):	**303.7**
Cash & cash eq. (USD m):	**236.3 *)**
Shares outstanding (m):	**21.43 (after offering)**
Volume:	**611,210**
Free float:	**31.3% **)**
52-week Range:	**8.29-35.50**

EUR mln	2012A	2013A	2014A
Revenues	-	2.943	4.685
Net Profit (Loss)	(14.716)	(26.820)	(37.040)
Net loss per share (cents)	(1.70)	(2.48)	(2.16)
R&D costs	(10.231)	(13.182)	(33.932)
Cash increase/(decrease)	(0.837)	23.535	23.581
Cash and marketable sec.	0.263	23.810	53.219

**) Includes the short term cash inflow from the deal with BMY and the net proceeds of the public offering*

***) Current major shareholders, management and additional 4.9% equity stake of BMY*

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- uniQure (NASDAQ:QURE) is a Dutch based biotechnology company that is developing gene therapies in liver-based diseases, cardio/metabolic diseases and CNS diseases using its proprietary technology platform. Its transgene delivery system is based on common, adeno-associated viruses, or AAV. In February 2014, uniQure completed IPO on NASDAQ, placing 5,400,000 shares at USD 17 per share, raising a total of USD 91.8 million.

- Its lead product Glybera is a gene therapy product for lipoprotein lipase deficiency (LPLD), a debilitating disease potentially resulting in lethal pancreatitis. The EMA approved Glybera in November 2012 but the launch in the EU was delayed due to the fact that the company needed to collect six-year follow up data on its benefits. This month the company received a negative preliminary regulatory opinion from the EMA. Based on the six-year follow up data, the rapporteur designated by the Committee for Advanced Therapy Medicinal Products (CAT) concluded that Glybera lacks efficacy and its benefit-risk balance is negative. The report will be reviewed this week, followed by a CHMP meeting on April 21-23. The CHMP is the committee at the EMA that is responsible for preparing opinions on questions concerning medicines for human use.

- We estimate there are 150-200 eligible patients in Europe where partner Chiesi will market the drug, paying 20-30% royalties to uniQure. The same number of patients apply for the US. We expect peak sales (>2020) of USD 30-40 million. As a result we value Glybera to be USD 2-3 per share, also taking into account a much slower EU launch (2016) and potential US launch (2019). This is a considerable lower valuation than most US analysts calculate. We feel that, although Glybera is the world's first approved gene therapy product, it is of little commercial value for uniQure and therefore to its shareholders. Besides, Glybera can expect future competition from Novartis' small molecule pradigastat against pancreatitis, which is currently in Phase III.

- Last week, uniQure surprised with the news that it has entered into an agreement with Bristol Myers Squibb (BMY) that provides BMY exclusive access to its gene therapy platform for multiple targets in cardiovascular diseases. The collaboration includes uniQure's proprietary gene therapy program for congestive heart failure (CHF) that is intended to restore the heart's ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and thereby improve clinical outcomes for patients with reduced ejection fraction. (CHF) is a rapidly progressing and in many cases life-threatening disease affecting 26 million people worldwide. BMY is paying uniQure USD 50 million in cash and at least USD 32 million in equity up front (BMY is buying 4.9% of uniQure's shares at USD 33.84 per share). The company will pay another USD 15 million within three months when three gene therapy targets are selected. BMY has agreed to buy another 5% equity in uniQure by the end of the year at a 10% premium, along with warrants for yet another 10% of the company at a premium based on more targets being introduced into the deal. uniQure could also get USD 254 million in future payments for the congestive heart failure program, and USD 217 million for each other gene therapy to come from the deal.

- This month, US gene therapy company Celladon (CLDN) is expected to report topline data from its CUPID2 Phase IIb trial of lead product MYDICAR. MYDICAR is a gene therapy for the treatment of advanced heart failure (HF) that like uniQure is using AAV as its vector. The data release has been eagerly anticipated since the completion of patient enrollment in March 2014. The primary endpoint of this study is time to recurrent HF-related hospitalization, which was accepted by the FDA in 2012 in a Special Protocol Assessment as an acceptable endpoint for pivotal trials. Secondary endpoints include time-to-first terminal event, symptoms, exercise capacity, quality of life, and safety. In April 2014, the program received Breakthrough Therapy Designation from the FDA. Positive results have the

potential to be a major catalyst for growth for Celladon and it will also have an impact on uniQure as a validation of its own AAV based platform.

- As of 31 December 2014, the company had USD 57 million in cash. The agreement with BMY brings in another USD 97 million within 3 months. Next to that, following the BMY deal, uniQure announced it commences a secondary public offering of 3 million ordinary shares at USD 29.50 per share. This all adds up to a total of USD 236 million in cash and cash equivalents. Last week, management stated that the expected cash burn for 2015 is EUR 45-55 million (USD 50-60 million), which includes the start of a Phase I/II study of AMT-060 in Hemophilia B, preparing a US pivotal study of Glybera and the GMP validation of the Lexington facility.

- Based on our sum-of-the-parts valuation, we believe **uniQure** is well priced at the current share price of USD 28.44. Using our NPV-based valuation model, we estimate the company's total value to be in the range of USD 500-550 million, or USD 23-25 per share. We attribute USD 3 to Glybera, including the EU royalties and the Glybera US franchise and assume a 50% probability of success. Its Hemophilia B program AMT-060 in Phase I/II, we value at USD 10 with a 20% probability of success. We value its partnership with BMY in CVD at USD 10 with a 10% probability of success, as this is a preclinical program still. We feel that gene therapy company Celladon (CLDN) with its MYDICAR program in CVD in late stage development is much lower priced, considering that both companies have comparable technology platforms. We therefore prefer to invest in Celladon compared to uniQure, keeping in mind the expected release of the Phase IIb heart failure data later this month.

Analyst: Marcel Wijma MSc

Marcel Wijma, Chief Research Officer and managing partner, has a longstanding history in financial biotech research. After selling Van Leeuwenhoeck Research (VLR) to SNS Securities in 2006, he established an award winning analyst team in biotech/life sciences at SNS Securities. In 2009, Marcel was awarded by Financial Times/Starmine as being one of the Top-3 biotech analysts in Europe. Later that year, Marcel purchased VLR from SNS Securities after which the company was reconstituted. At VLR, he leads the professional VLR research organisation, which is augmented by selected external financial researchers with a specialisation in Life Sciences. Mr. Wijma has a Masters degree in Financial Economics from Erasmus University in Rotterdam.

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