



uniQure Announces Results for Financial Year 2013

Amsterdam, the Netherlands, March 26, 2014 — uniQure N.V. (Nasdaq: QURE), a leader in human gene therapy, today announced unaudited results for the financial year ended December 31, 2013. As of December 31, 2013, the Company held cash and cash equivalents of €23.8 million.

Corporate Highlights

2013 represented an important year for uniQure, with a substantially strengthened financial position and encouraging advances in the Company's development programs. This was in advance of the successful completion in February 2014 of uniQure's IPO on NASDAQ, placing 5,400,000 shares at \$17 per share, raising a total of gross \$91.8 million (€67.3 million) and after commissions but before expenses a net of \$85.4 million (€62.6 million).

The following key achievements were completed during 2013:

- Two collaboration agreements signed with Chiesi Farmaceutici S.p.A. (Chiesi), one for the commercialization in the EU and selected additional territories of Glybera® for LPLD and the second for the co-development and commercialization of uniQure's hemophilia B program in the EU and additional territories (uniQure retains full rights for both programs in North America, Japan and certain other territories)
- Investment by Chiesi of €14 million in new uniQure equity
- Initial development of a 55,000 square foot (5,100 m²) manufacturing facility in Lexington, Massachusetts, USA
- Additional 2013 financings that included the extension and conversion of a €13.5 million convertible loan facility and a new \$10 million (€7.4 million) venture debt facility
- Advanced preparations to initiate a Phase 1/2 clinical study for hemophilia B with Chiesi
- Initiation by collaborator Institut Pasteur of a Phase 1/2 clinical study for Sanfilippo B Syndrome
- Announcement by collaborator UCSF together with the NIH of a Phase 1 clinical study in Parkinson's disease
- Completion of recruitment for a Phase 1 clinical study for acute intermittent porphyria with collaborator Digna Biotech

Commenting on these achievements, Jörn Aldag, uniQure Chief Executive Officer, said: "We have successfully transformed uniQure over the last 18 months into a leader in the field of gene therapy. The business is now well positioned to continue delivering on the promise of gene therapy through the commercial launch of our first product, Glybera, advance of our clinical pipeline, and continued progress with our research activities. We look forward to key events expected in the next several months, including full analysis of the long-term Glybera data, launch of Glybera in the EU and further progress for our pipeline."

Regarding webcast of company results and expectations, uniQure management will conduct biannual investor conference calls/webcasts, the first of which will occur mid-2014.

Financial Highlights

Licensing and collaboration revenues for the 12 months ended December 31, 2013 were €2.9 million, compared with zero revenues in 2012. The majority of 2013 revenues are related to development activities that were reimbursable by Chiesi under the Company's co-development agreement for hemophilia B.

Research and development expenses were €13.2 million for the 12 months ended December 31, 2013, compared to €10.2 million for the comparable period in 2012. The increase reflected the expansion of research and development activities to support the further development of the hemophilia B program as well as the further development of other pipeline product candidates. The amount of research and development expenses is shown net of charges that were capitalized in relation to the development of the Company's approved product, Glybera.

Selling, general and administrative expenses were €11.6 million for the 12 months ended December 31, 2013, compared to €4.6 million in 2012. The increase resulted principally from increased headcount in 2013 as the Company continued to ramp up operations following a strategic restructuring at the end of 2011 and increased commercial, legal and other advisory fees.

Net loss for the full year 2013 was €26.8 million or €2.48 per share, compared to €14.7 million or €1.70 per share for the full year 2012.

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

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 risk of cessation or delay of any of the ongoing or planned clinical studies and/or development of our product candidates, the risk of delay or failure to successfully commercialize or obtain further regulatory approval of Glybera, and the risk that our collaborations with Chiesi or our other collaboration partners will not continue or will not be successful. 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, regulatory oversight, product commercialization, intellectual property claims, and the risks, uncertainties and other factors described under the heading “Risk Factors” in uniQure’s prospectus filed with the Securities and Exchange Commission dated February 5, 2014. 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.

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UNIQUE N.V.

Consolidated Statements of Comprehensive Income
(€ in thousands, except share and per share data)

	Years Ended December 31	
	2012	2013 (unaudited)
License revenues	—	440
Collaboration revenues	—	2,503
Total revenues	—	2,943
Cost of goods sold	—	(800)
Gross profit	—	2,143
Other income	649	585
Research and development expenses	(10,231)	(13,182)
Selling, general and administrative expenses	(4,564)	(11,628)
Other losses, net	(45)	(453)
Total operating costs	(14,840)	(25,263)
Operating result	(14,191)	(22,535)
Finance income	22	102
Finance expense	(547)	(4,387)
Finance income/(expense)—net	(525)	(4,285)
Result before corporate income taxes	(14,716)	(26,820)
Corporate income taxes	—	—
Net loss	(14,716)	(26,820)
Other comprehensive income	—	12
Total comprehensive loss*	(14,716)	(26,808)
Loss per share attributable to the equity holders of the Company during the year		
Basic and diluted loss per share	(1.70)	(2.48)

* Total comprehensive loss is fully attributable to equity holders of the group

UNIQURE N.V.

Consolidated Balance Sheets
(€ in thousands)

	As of December 31,	
	2012	2013 (unaudited)
Assets		
Non-current assets		
Intangible assets	3,278	7,775
Property, plant and equipment	1,185	2,614
Other non-current assets	—	923
Total non-current assets	4,463	11,312
Current assets		
Receivables from related parties	26	1,425
Trade and other receivables	815	1,557
Inventories	—	865
Cash and cash equivalents	263	23,810
Total current assets	1,104	27,657
Total assets	5,567	38,969
Equity		
Share capital	483	610
Share premium	114,795	142,459
Other reserves	1,508	6,536
Accumulated deficit	(117,234)	(144,041)
Total equity	(448)	5,564
Liabilities		
Non-current liabilities		
Borrowings	—	6,292
Financial lease liabilities	450	302
Deferred rent	—	680
Deferred revenue	—	15,679
Total non-current liabilities	450	22,953
Current liabilities		
Trade and other payables	4,067	7,601
Debt to related party—financial liability	1,366	633
Debt to related party—embedded derivative	132	722
Borrowings—embedded derivative	—	217
Deferred revenue	—	1,279
Total current liabilities	5,565	10,452
Total liabilities	6,015	33,405
Total equity and liabilities	5,567	38,969