FOR IMMEDIATE RELEASE

uniQure Announces Results for the Second Quarter and First Half of 2014 and Provides Update on Gene Therapy Programs

Amsterdam, the Netherlands, September 2, 2014 — uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced results for the second quarter of 2014 and an update on multiple gene therapy programs.

Corporate Highlights

- In July, uniQure announced the acquisition of InoCard GmbH, an innovative, early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy to preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF), a rapidly progressing disease affecting 26 million people worldwide. InoCard founders Prof. Patrick Most and Prof. Hugo Katus will join uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board for Cardiovascular Diseases, respectively.
- uniQure closed an additional $10 million venture debt loan with Hercules Technology Growth Capital, Inc., increasing the total loan amount to $20 million and providing the Company with greater balance sheet strength and flexibility. The additional capital will be devoted both to rapidly advancing uniQure's pipeline and to accessing early-stage opportunities that will enable the Company to leverage its gene therapy platform and manufacturing expertise. As of June 30, 2014, the Company held cash and cash equivalents of €72.1 million.
- Construction has been completed on the Lexington, Massachusetts facility and employees have moved in after an occupancy certificate was received for the GMP rooms in June 2014. The facility is expected to be fully operational in the first half of 2015.
- Will Lewis, MBA/JD, joined uniQure's Supervisory Board. Mr. Lewis is President and Chief Executive Officer of NASDAQ listed Insmed Inc. and brings to uniQure more than 20 years of executive experience in the pharmaceutical and finance industries as well as in the field of orphan diseases.

Pipeline Program Updates

- **Hemophilia B Program** - The Company remains on target for the initiation of a Phase 1/2 clinical trial for the hemophilia B AAV5 candidate late in the second half of 2014. Manufacturing of clinical material is currently being completed to enable release as planned in Q4 2014. uniQure has submitted the necessary documentation for the clinical trial application to the authorities in Germany.
- **Collaborator-sponsored Pipeline Programs -** The current clinical trials being conducted in the Sanfilippo B program with Institut Pasteur and the Acute Intermittent Porphyria program with University of Navarra are fully enrolled and on track for 2014 (AIP) and 2015 (Sanfilippo B) data release. In the partnership with UCSF and NIH for Parkinson's disease five of the six patients in the first dose cohort have been dosed.
- **AAV5 Vector Validation -** In May, uniQure successfully documented the potential clinical utility of its proprietary AAV5 vector for liver-directed gene therapy. Results obtained from an ongoing Acute Intermittent Porphyria (AIP) dose-escalation Phase 1 trial provided safety evidence for the AAV5 vector from the baculovirus production platform by successfully delivering DNA to the liver in AIP patients without liver enzyme perturbations.
- **4D Molecular Therapeutics Collaboration –** In the research program with 4D Molecular Therapeutics, under which uniQure gained exclusive access to 4D's AAV vector discovery and optimization technology for gene delivery to the central nervous system and liver, the Company expects to make a preliminary selection of new synthetic vectors in the first half of 2015.

Glybera Updates

- **European Launch** – In early August uniQure and its commercialization partner Chiesi provided an update on preparations relating to the launch of Glybera® (alipogen tiparvovec) in the European Union for the treatment of the orphan disease lipoprotein lipase deficiency (LPLD). Chiesi has exclusive rights to commercialize Glybera in the EU and selected additional territories. Chiesi and uniQure decided to include the six-year follow-up pancreatitis data from the study AMT 011-05, in the pricing and reimbursement applications as announced on June 3, 2014. Chiesi now expects to launch Glybera in the fourth quarter of 2014/first quarter of 2015.

- **Six-year Follow-up Data -** uniQure announced the analysis of six-year follow-up data from the study AMT 011-05 for Glybera for the treatment of LPLD, which validate long-term clinical benefits. Following Glybera treatment LPLD patients no longer experienced severe pancreatitis, and the occurrence of less severe events was reduced by approximately 50%.
- **U.S. Regulatory Progress** – To optimize the time for patients in the US to access Glybera and make the overall process more efficient, uniQure intends to combine both EMA and FDA requirements into one clinical protocol for the Phase 4 study. As a result, the Company expects a delay in generating the clinical data to support the filing in the US. This delay is in part the result of longer than expected EMA approval timelines due to required changes in the protocol in support of the US filing strategy, resulting in a planned Phase 4 trial start mid 2015, and in part the result of limited product supply due to a Glybera-specific batch release assay being out of specification.
- **Manufacturing Ramp-up** - uniQure continues to optimize its original Glybera-specific manufacturing process to match the higher manufacturing standards already achieved with later pipeline products (e.g. Acute Intermittent Porphyria, Sanfilippo, Hemophilia) and to increase Glybera-specific batch release success rates. The Company expects to be able to meet 2015 EU commercial demand for Chiesi with current stock and from future production runs commencing later in 2014, assuming higher manufacturing standards are successfully implemented.
- **New Distribution Alliance** - uniQure signed an exclusive distribution agreement with Medison Pharma Ltd., Israel's leading pharmaceutical marketing group. Under the terms of the agreement, Medison will market Glybera in Israel and the Palestinian Authority territories. uniQure continues to negotiate similar marketing agreements for Glybera in those regions where the Company plans to market the product with a commercialization partner.

Jörn Aldag, uniQure Chief Executive Officer, commented: "uniQure is steadily executing its strategy of building a valuable clinical and pre-clinical pipeline from its gene therapy platform. We made major progress toward the achievement of preclinical, clinical, and corporate development goals, which we view to be essential to maintain our position at the forefront of gene therapy. In the second half of the year, we are focused on the start of the hemophilia B clinical trial."

Financial Highlights
As of June 30, 2014, the Company held cash and cash equivalents of €72.1 million. The numbers for the six months ended June 30, 2013 as presented below, have been restated; for further information please see the financial statements appearing at the end of this release.

Licensing and collaboration revenues for the three months ended June 30, 2014 were €1.0 million, compared with €0.8 million in the same period of 2013. For the six months ended June 30, 2014, total revenue was €2.2 million, compared to €0.8 million in the first six months in 2013. Collaboration revenues represent development activities that are reimbursable by Chiesi under the Company's co-development agreement for hemophilia B. License revenues represent the monthly amortization of the upfront payments received under the Chiesi agreements entered into in June 2013.

Research and development expenses were €8.0 million for the three months ended June 30, 2014, compared to a restated €2.9 million for the same period in 2013. Research and development expenses for the six months ended June 30, 2014 are €14.2 million compared to €6.4 million for the same period in 2013. The increase reflected the expansion of research and development activities to support the further development of the hemophilia B program, the further development of other pipeline product candidates, as well as the company's efforts to maintain its leadership position in the gene therapy field. 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.

Net loss for the three months ended June 30, 2014 was €9.0 million or €0.51 per share, compared to €7.8 million or €0.80 per share for the same period in 2013. Net loss for the six months ended June 30, 2014 was €16.8 million or €1.03 per share, compared to €13.0 million or €1.33 per share for the same period in 2013.

In the three months ended June 30, 2014 the Company signed an amended and restated loan agreement with Hercules Technology Growth Capital Inc. to increase its existing venture debt facility to $20.0 million (€14.6 million) and in May 2014 the Company rolled out the 2014 Option Plan granting a total of 926,000 options to staff and affiliates, with an exercise price of $9.35.

For further financial information for the period ending June 30, 2014, please refer to the financial statements appearing at the end of this release.

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 commercial launch of Glybera in the EU, 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 our products, and the risk that our collaborations 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, manufacturing processes and facilities regulatory oversight, product commercialization, intellectual property claims, 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 25, 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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Unaudited Condensed Consolidated Balance Sheets
(€ in thousands)

	DECEMBER 31, 2013	JUNE 30, 2014
Assets		
Non-current assets		
Intangible assets	7,775	9,728
Property, plant and equipment	2,614	14,614
Other non-current assets	923	932
Total non-current assets	11,312	25,274
Current assets		
Receivables from related parties	1,425	1,453
Trade and Other Receivables	1,557	2,513
Inventories	865	427
Cash and cash equivalents	23,810	72,057
Total current assets	27,657	76,450
Total assets	38,969	101,724
Equity		
Share capital	610	880
Share premium	142,459	204,142
Other reserves	6,536	11,162
Accumulated deficit	(144,041)	(160,872)
Total equity	5,564	55,312
Liabilities		
Non-current liabilities		
Borrowings	6,292	14,498
Financial lease liabilities	302	219
Deferred rent	680	5,247
Deferred revenue	15,679	15,238
Total non-current liabilities	22,953	35,202
Current liabilities		
Trade and other payables	7,601	9,178
Debt to related party - derivative	722	516
Borrowings	633	—
Borrowings - derivative	217	170
Deferred rent	—	3
Deferred revenue	1,279	1,343
Total Current Liabilities	10,452	11,210
Total liabilities	33,405	46,412
Total equity and liabilities	38,969	101,724

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Unaudited Condensed Consolidated Statements of Comprehensive Loss
(€ in thousands, except share and per share data)

	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2013 (as restated)	2014	2013 (as restated	2014
License revenues	—	221	—	441
Collaboration revenues	758	821	758	1,771
Total revenues	758	1,042	758	2,212
Cost of goods sold	(800)	—	(800)	—
Other income	203	152	391	390
Research and development expenses	(2,852)	(8,008)	(6,421)	(14,226)
Selling, general and administrative expenses	(2,437)	(2,548)	(4,157)	(4,817)
Other gains / losses, net	26	583	35	64
Total Operating Costs	(5,060)	(9,821)	(10,152)	(18,589)
Operating result	(5,102)	(8,779)	(10,194)	(16,377)
Finance income	—	44	44	71
Finance expense	(2,682)	(255)	(2,814)	(514)
Finance income/(expense)—net	(2,682)	(211)	(2,770)	(443)
Result before corporate income taxes	(7,784)	(8,990)	(12,964)	(16,820)
Corporate income taxes	—	—	—	—
Net Loss	(7,784)	(8,990)	(12,964)	(16,820)
Items that may be subsequently reclassified to profit or loss	—	(11)	—	(10)
Other comprehensive income	—	—	—	(10)
Total comprehensive loss*	(7,784)	(9,001)	(12,964)	(16,830)
Loss per share attributable to the equity holders of the Company during the year				
Basic and diluted loss per share	(0.80)	(0.51)	(1.33)	(1.03)

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

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Unaudited Condensed Consolidated Statement of Changes in Equity/Deficit
(€ in thousands)

	TOTAL SHARE CAPITAL	SHARE PREMIUM	OTHER RESERVES	ACCUMULATED DEFICIT	TOTAL EQUITY/DEFICIT
Balance at January 1, 2013	483	114,795	1,508	(117,234)	(448)
Result for the period				(12,964)	(12,964)
Capital contributions	4	274			278
Share based payment/expense			947		947
Balance at June 30, 2013 (as restated)	487	115,069	2,455	(130,198)	(12,187)
Result for the period				(13,856)	(13,856)
Other Comprehensive Income				13	13
Capital contributions	123	27,390			27,513
Result on conversion of the Loan			3,005		3,005
Share-based payment/expense			1,076		1,076
Balance at December 31, 2013	610	142,459	6,536	(144,041)	5,564
Result for the period				(16,820)	(16,820)
Other Comprehensive Income				(10)	(10)
Proceeds from shares issued	270	62,351			62,621
Share issuance cost		(668)			(668)
Share-based payment/expense			4,626		4,626
Balance at June 30, 2014	880	204,142	11,162	(160,872)	55,312

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Unaudited Condensed Consolidated Statement of Cash Flows
(€ in thousands)

	SIX MONTHS ENDED JUNE 30,	
	2013 (as restated)	2014
Cash flow from operating activities		
Result before corporate income tax	(12,964)	(16,820)
Adjustments for:		
—Depreciation	259	310
—Lease Incentive	—	3,876
—Derivative result	1,954	(253)
—Exchange result	(35)	(64)
—Other non-cash items	800	(9)
—Share-based payment expenses	947	4,626
—Changes in other non-current assets	—	—
—Changes in trade and other receivables	(17,845)	(292)
—Movement in inventories	(188)	438
—Changes in trade and other payables	97	(1,240)
—Changes in deferred revenue and provisions	17,083	(377)
—Movement in other liabilities	469	448
—Interest (income)/expense	613	650
Cash used in operations	(8,810)	(8,707)
Interest paid	(6)	(461)
Net cash used in operating activities	(8,816)	(9,168)
Cash flow from investing activities		
Purchases of property, plant and equipment	(324)	(9,787)
Purchases of intangible assets	(1,225)	(1,953)
Interest received	—	59
Net cash used in investing activities	(1,549)	(11,681)
Cash flow from financing activities		
Capital contribution from shareholders	278	
Proceeds from shares issued	—	62,621
Share issuance cost	—	(668)
Convertible loans drawn down	11,999	—
Exchange result on Borrowings	—	46
Proceeds from borrowings	7,492	7,184
Redemption of financial lease	(70)	(77)
Repayments of borrowings	—	—
Net cash generated from financing activities	19,699	69,106
Net increase in cash, cash equivalents, and other bank overdrafts	9,334	48,257
Currency effect cash and cash equivalents	—	(10)
Cash, cash equivalents, and other bank overdrafts at beginning of the period	263	23,810
Cash, cash equivalents, and other bank overdrafts cash at end of the period	9,597	72,057