uniQure

CORRECTING and REPLACING -- uniQure Announces Results for Fourth Quarter and Financial Year 2015

April 4, 2016

AMSTERDAM, the Netherlands, April 4, 2016 (GLOBE NEWSWIRE) -- In a release issued under the same headline earlier today by uniQure N.V. (NASDAQ:QURE), the tables were not included. The corrected release follows:

uniQure N.V. (NASDAQ:QURE), a leader in human gene therapy, today announced audited results for the fourth quarter and year ending December 31, 2015, and provided a corporate overview on its pipeline programs and operations.

"Over the past several months, we announced proof-of-concept results from our Phase I/II clinical trials in Sanfilippo B and hemophilia B. Both studies demonstrated not only encouraging safety and efficacy data, but also validated our proprietary AAV5 technology and insect cell, baculovirus production system in both the brain and the liver," said Dan Soland, CEO of uniQure. "In 2016, we will move closer to advancing these lead programs into pivotal studies and initiate Investigational New Drug (IND)-enabling studies on additional product candidates that are expected to enter human clinical trials in 2017. We are particularly looking forward to sharing longer follow-up data from our ongoing Phase I/II clinical study of AMT-060 in hemophilia B and from the Sanfilippo B patients. Since joining uniQure in December, I am increasingly convinced that our validated technology platform, commercial-scale manufacturing capabilities and broad clinical and preclinical programs will continue to drive our leadership in gene therapy."

Corporate Highlights

Liver/Metabolism Therapeutic Focus Area

- **Reported Encouraging Top-line Data from Low-Dose Cohort in Phase I/II Study of AMT-060** – On January 7, 2016, uniQure announced preliminary topline results from the first, low-dose cohort in the ongoing AMT-060-01 Phase I/II Hemophilia B study. Initial results showed that AMT-060 was generally well tolerated and the first two patients that completed at least 12 weeks of follow-up showed promising Factor IX expression levels of 5.5% and 4.5% of normal. Additionally, four out of five patients discontinued recombinant FIX prophylaxis as of January 6, 2016. These early data validate successful transduction of the liver using uniQure's proprietary AAV5 vector. uniQure expects to report results on all five patients from the low-dose cohort at a scientific conference in the second quarter of 2016.
- **Initiated Dosing of AMT-060 in High-Dose Cohort** – On March 14, 2016, the Company announced that the first patient in the high-dose cohort of the AMT-060-01 trial had been treated. As of today, 2 patients in the high-dose cohort have been treated. All 8 patients screened so far in the Phase I/II trial have tested negative for anti-AAV5 antibodies.

CNS Therapeutic Focus Area

- **Positive Results from Phase I/II Study of AMT-110 in Sanfilippo B** – On September 19, 2015, encouraging results from an academic-sponsored Phase I/II trial in Sanfilippo B using uniQure's novel AAV5-based gene therapy were presented at the European Society of Gene and Cell Therapy (ESGCT) in Helsinki, Finland. In all four patients, researchers verified the restoration of catalytical activity of the NaGlu protein in the cerebrospinal fluid (CSF) from 0% at baseline up to 14-17% of normal at three months and maintained further at 12 months. The trial also demonstrated that incremental cognitive development was maintained in all four patients with no progression of brain atrophy detected via MRI scans. These data validate the effective transmission of the NaGlu gene into the brain with uniQure's proprietary AAV5 viral vector. uniQure expects to present 30-month follow-up data from the four patients in early 2017.
- **Sponsorship of Extension Protocol for Phase I/II Study of AMT-110 to Transition to uniQure** – In January 2016, uniQure and the academic consortium comprised of Institut Pasteur, the French Muscular Dystrophy Association, Vaincre les Maladies Lysosomales and Institut National de la Santé et de la Recherche Médicale (INSERM), executed a term sheet reflecting the license of certain data and intellectual property from the consortium-sponsored Phase I/II study of AMT-110 in Sanfilippo B. uniQure and the consortium are currently negotiating the terms of a definitive agreement. Additionally, uniQure has assumed the sponsorship of the Phase I/II extension study, enabling the Company to continue the follow-up of the four patients treated to date.
- **Phase I Study in Parkinson's Disease to Complete Enrolling Second Cohort** – A Phase I clinical study in Parkinson's disease led by Krystof Bankiewicz, MD, PhD of the University of California at San Francisco together with John D. Heiss, MD, and colleagues from the National Institutes of Heath, using an AAV-glial cell line-derived neurotrophic factor (GDNF) product licensed from AMGEN for gene therapy applications, has completed enrollment of its first of four six-patient cohorts and is expected to complete the treatment of the second cohort later in the year.
- **Preclinical Proof-of-Concept Achieved in Huntington's Disease** – On March 22, 2016, uniQure announced the publication of preclinical data in the March 2016 edition of *Molecular Therapy-Nucleic Acids* which showed that AMT-130, a novel AAV5-based gene therapy candidate for Huntington's disease, achieved preclinical proof-of-concept. The studies, which were conducted *in vitro* and in a humanized mouse model, demonstrated silencing of the mutated Huntingtin gene with therapeutic microRNAs delivered via an AAV5 vector. uniQure has selected its lead candidate and has initiated IND-enabling studies.

Cardiovascular Therapeutic Focus Area

- **Four Targets Designated by BMS**; **Advancing S100A1 Gene Therapy Towards IND Filing** – On April 6, 2015, uniQure announced a collaboration with Bristol-Myers Squibb in which the two companies would develop gene therapies for cardiovascular diseases. In total, the parties may collaborate on up to ten targets, of which four have been designated. uniQure and BMS are currently conducting safety and toxicology studies for the S100A1 gene therapy using uniQure's proprietary insect-cell, baculovirus expression system and are advancing towards an IND filing.

Technology and Manufacturing

- **Lexington Facility Operational and in Process of Ramping Up to Commercial-Scale Production** – In 2015, uniQure completed the build-out of its 53,000 sq. ft. fully scalable gene therapy manufacturing plant in Lexington, Massachusetts. The facility is operational and currently producing research batches. Scale-up of AMT-060 production is currently underway and expected to be completed in 2016 in order to supply clinical GMP material for a pivotal study in hemophilia B.
- **Designation of AAV Capsid Variants with 4D; Initiating Preclinical Validation** – In the fourth quarter of 2015, uniQure designated specific synthetic AAV capsid variants derived from 4D's proprietary capsid library for further improvement of efficacy in both the CNS and Liver/Metabolism Therapeutic Focus Areas. uniQure will initiate preclinical validation of the designated capsid variants in non-human primates in 2016.
- **Designation of Synthetic Liver-Specific Promoters from Synpromics Ltd; Initiating Preclinical Validation**– As part of the cooperation and license agreement signed in January 2015, Synpromics delivered a series of synthetic liver-specific promoters derived from their rationally-designed promoter library technology. uniQure will use these promoters to further improve the therapeutic activity of AAV-vectors in the liver. uniQure will initiate preclinical testing of these promoters in non-human primates during 2016.

Corporate Finance

- **Strong Financial Position** – As of December 31, 2015, uniQure had €203.5 million of cash on hand, which is expected to fund operations into the second half of 2018. To date, uniQure has received approximately $140 million in upfront and other consideration from BMS. In addition, on April 7, 2015, uniQure closed a follow-on public offering of 3,000,000 ordinary shares at an offering price of $29.50 per share raising a total of $83.2 million.

Human Resources

- **Expanding Key Talent** – Over the course of 2015 uniQure has strengthened its management team with experienced industry leaders in its two key locations in Lexington, Massachusetts and Amsterdam, including the appointment of Dan Soland as CEO in December 2015, Matt Kapusta as CFO in January 2015, Dr. Charlie Richard as SVP, R&D Neuroscience in July 2015 and Dr. Deya Corzo, SVP, R&D Liver/Metabolism in July 2015.

Financial Highlights

As of December 31, 2015, the Company held cash and cash equivalents of €203.5 million, compared with €53.2 million as of December 31, 2014. The increase was due to the consideration received from BMS during the period and the Company's follow-on offering in April 2015, offset in part by cash used in research, development and general corporate activities.

Revenue for 2015 was €9.4 million, compared with €4.7 million in 2014. These revenues are primarily related to the Company's collaboration agreements with Bristol-Myers Squibb and Chiesi, as well as Glybera product sales, which commenced in the third quarter of 2015.

Cost of goods sold in 2015 was €0.6 million, compared with zero in 2014. Cost of goods sold includes the cost of Glybera product sales and amortization of specific Glybera-related license agreements, which were required to be expensed upon the initiation of commercialization in the third quarter of 2015.

Research and development expenses were €46.8 million in 2015, compared to €33.9 million in 2014. The increase is related to the continuation of uniQure's Phase I/II clinical study of AMT-060 in hemophilia B, the continued progression of uniQure's other product candidates and increased activity in the Company's U.S. facility.

Selling, general and administrative expenses were €19.3 million in 2015, compared with €11.2 million in 2014. The increase was primarily due to expenses related to consultants and professional fees associated with business development, expenses related to the Company's follow-on offering conducted in April 2015 and other general and administrative activities.

Other gains/losses were a loss of €0.2 million for 2015, compared to a gain of €5.8 million in 2014. The loss was primarily attributable to the impact of foreign currency exchange rates on the Company's dollar-denominated deposits.

In the third quarter of 2015, the Company incurred a one-time, non-recurring impairment charge on certain Glybera-related intangible assets of €11.6 million related to the revision of the Company's forecasted number of patients treated with Glybera and certain reimbursement-related developments in Europe.

The net loss for the fourth quarter of 2015 was €14.1 million, or €0.58 per share, compared with €11.2 million, or €0.62 per share, for the fourth quarter of 2014. The net loss for the full years 2015 and 2014 was €71.5 million, or €3.24 per share and €37.0 million, or €2.16 per share, respectively.

For further financial information for the period ending December 31, 2015, please refer to the financial statements appearing at the end of this release.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. We are leveraging our modular and validated technology platform to rapidly advance a pipeline of proprietary and partnered gene therapies to treat patients with CNS, liver/metabolic and cardiovascular diseases.www.uniQure.com

uniQure 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 development of our gene therapiesthe success of our collaborations, , and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with collaboration arrangements, our and our collaborators' clinical development activities, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's 2014 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 25, 2014 and its 2015 Annual Report on Form 20-F to be filed with the Securities and Exchange Commission on or about the date hereof. 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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Consolidated Statements of Financial Position
(€ in thousands)

	NOTE	DECEMBER 31, 2014	DECEMBER 31, 2015
Assets			
Non-current assets			
Goodwill	2, 5, 6	1,342	442
Intangible assets other than Goodwill	5, 6	16,368	7,209
Property, plant and equipment	7	19,667	23,820
Other non-current assets	8	1,022	1,142
Total non-current assets		38,399	32,613
Current assets			
Receivables from related parties	9	2,426	3,792
Trade and other receivables	9	1,542	1,730
Inventories	10	200	435
Cash and cash equivalents	11	53,219	203,532
Total current assets		57,387	209,489
Total assets		**95,786**	**242,102**
Equity			
Share capital		905	1,216
Share premium		206,111	344,803
Other reserves		17,149	26,026
Accumulated deficit		(181,081)	(252,561)
Total equity	**12**	**43,084**	**119,484**

Liabilities			
Non-current liabilities			
Borrowings	14	16,418	13,434
Derivative financial instruments- related parties	14	--	530
Financial lease liabilities	15	134	--
Deferred rent	27	5,658	5,737
Deferred revenue	17	15,387	75,852
Deferred tax liabilities	5, 23	1,379	--
Contingent considerations	5	1,454	2,687
Total non-current liabilities		**40,430**	**98,240**
Current liabilities			
Trade and other payables	15, 16	9,617	11,220
Derivative financial instruments - related parties	14	645	992
Borrowings	14	--	5,124
Borrowings - derivative	14	207	238
Deferred rent	27	475	579
Deferred revenue	17	1,328	6,225
Total current liabilities		**12,272**	**24,378**
Total liabilities		**52,702**	**122,618**
Total equity and liabilities		**95,786**	**242,102**

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Consolidated Statements of Comprehensive Loss

(€ in thousands, except share and per share data)

		TWELVE MONTHS ENDED DECEMBER 31,		
	NOTE	**2013**	**2014**	**2015**
License revenues	17	440	883	2.854
Collaboration revenues	17	2.503	3.802	6.271
Product sales	17	--	--	300
Total revenues		**2.943**	**4.685**	**9.425**
Cost of goods sold	18	(800)	--	(584)
Other income	21	585	773	708
Research and development expenses	18, 19	(13.182)	(33.932)	(46.781)
Selling, general and administrative expenses	18, 20	(11.628)	(11.167)	(19.317)
Impairment of intangible assets	6	--	--	(11.640)
Other gains / losses, net		(453)	5.807	(248)
Total operating costs		**(25.478)**	**(38.519)**	**(77.862)**
Operating result		**(22.535)**	**(33.834)**	**(68.437)**
Finance income		102	254	549
Finance expense	22	(4.387)	(3.46)	(4.023)
Finance income/(expense)---net		**(4.285)**	**(3.206)**	**(3.474)**
Result before corporate income tax		**(26.82)**	**(37.04)**	**(71.911)**
Corporate income taxes	23	--	--	431
Net loss		**(26.82)**	**(37.04)**	**(71.480)**
Items that may be subsequently reclassified to profit or loss				
Currency translation differences on foreign operations		12	1.149	1.25
Other comprehensive income/(loss)	24	12	1.149	1.25
Total comprehensive loss		**(26.808)**	**(35.891)**	**(70.230)**
Loss per share attributable to the equity holders of the Company during the year:				
Basic and diluted loss per share	**25**	**(2,48)**	**(2,16)**	**(3,24)**

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Consolidated Statements of Changes in (Deficit)/Equity

(€ in thousands)

	Note	**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		--	--	--	(26,820)	(26,820)
Other comprehensive income/(loss)	12	--	--	--	12	12
Total comprehensive loss		**--**	**--**	**--**	**(26,808)**	**(26,808)**
Capital contributions		127	27,664	--	--	27,791
Result on conversion of loan		--	--	3,005	--	3,005
Share based payment/expense	13	--	--	2,023	--	2,023
Balance at December 31, 2013		**610**	**142,459**	**6,536**	**(144,041)**	**5,564**
Result for the period		--	--	--	(37,040)	(37,040)
Other comprehensive income		--	--	1,149	--	1,149
Total comprehensive loss		**--**	**--**	**1,149**	**(37,040)**	**(35,891)**
Capital contributions	12	295	64,320	--	--	64,615
Share issuance costs		--	(668)	--	--	(668)
Share based payment/expense	13	--	--	9,464	--	9,464
Balance at December 31, 2014	**12**	**905**	**206,111**	**17,149**	**(181,081)**	**43,084**
Result for the period		--	--	--	(71,480)	(71,480)
Other comprehensive income	24	--	--	1,250	--	1,250
Total comprehensive loss		**--**	**--**	**1,250**	**(71,480)**	**(70,230)**
Capital contributions	12	311	139,304	--	--	139,615
Share issuance costs		--	(612)	--	--	(612)
Share based payment/expense	13	--	--	7,627	--	7,627
Balance at December 31, 2015	**12**	**1,216**	**344,803**	**26,026**	**(252,561)**	**119,484**

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Consolidated Statements of Cash Flows

(€ in thousands)

		YEARS ENDED DECEMBER 31,		
	NOTE	**2013**	**2014**	**2015**
Cash flow from operating activities				
Net loss		(26,820)	(37,040)	(71,480)
Adjustments for:				
Depreciation	7	535	1,539	3,982
Amortization on intangibles assets	6	--	--	460
Impairment of intangible assets	6	--	--	11,640
Change in deferred tax liability	23	--	--	(479)
Lease incentive	27	134	5,452	183
Loss/(gain) on derivatives	14	3,446	(87)	(440)

Loss/(gain) on foreign exchanges	14	49	(4,692)	1,332
Changes in contingent consideration		--	153	1,232
Share-based expenses	13	2,023	9,464	7,627
Changes in other non-current assets		(923)	--	--
Changes in trade and other receivables		(1,439)	(952)	(1,554)
Movement in inventories		(865)	664	(235)
Changes in other current liabilities		3,655	1,540	(1,199)
Changes in deferred revenue	17	16,958	(242)	65,361
Intial recognition of warrants	3	--	--	2,622
Cash (used in) / generated by operations		**(3,247)**	**(24,201)**	**19,052**
Interest paid		(889)	(1,224)	(1,878)
Net cash (used in) / generated by operating activities		**(4,136)**	**(25,425)**	**17,174**
Cash flow from investing activities				
Purchases of property, plant and equipment	7	(1,336)	(15,769)	(5,671)
Purchases of intangible assets	6	(4,652)	(3,367)	(2,940)
Interest received		17	148	91
Acquisition of businesses	5	--	(1,463)	--
Net cash used in investing activities		**(5,971)**	**(20,451)**	**(8,520)**
Cash flow from financing activities				
Proceeds from shares issued	12	14,294	63,097	138,342
Share issuance cost	12	--	(668)	(612)
Convertible loans drawn down		11,999	--	--
Proceeds from Borrowings	14	7,492	7,184	--
Payments of finance lease	16	(143)	(156)	(168)
Net cash generated from financing activities		**33,642**	**69,457**	**137,562**
Net increase in cash, cash equivalents and bank overdrafts		**23,535**	**23,581**	**146,216**
Currency effect cash and cash equivalents		12	5,828	4,097
Cash, cash equivalents and bank overdrafts at beginning of the period		263	23,810	53,219
Cash, cash equivalents and bank overdrafts at end of the period		**23,810**	**53,219**	**203,532**

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