uniQure Announces 2016 Financial Results and Provides Update on Company Progress

March 15, 2017

LEXINGTON, Mass. and AMSTERDAM, the Netherlands, March 15, 2017 (GLOBE NEWSWIRE) -- uniQure N.V. (NASDAQ:QURE), a leading gene therapy company developing transformative therapies for patients with severe medical needs, today announces its financial results for the year 2016 and provides an update on company progress.

"In 2016, we made significant progress across all of our core programs and implemented strategic changes that have strengthened our organization and enhanced focus on our key priorities," stated Matthew Kapusta, chief executive officer of uniQure. "We enter 2017 with a solid financial position and strong clinical data to advance our hemophilia B program into a Phase III study, as well as to progress our gene therapy candidates in Huntington's disease and congestive heart failure towards IND filings. We believe the achievement of these objectives will deliver meaningful value to shareholders."

Recent Highlights from 2016

- **Promising Updated Phase I/II Data Presented on AMT-060 at ASH**

  - Updated results on AMT-060, including up to 52 weeks of follow-up on the first patient cohort receiving $5x10^{12}$ gc/kg and up to 26 weeks of follow-up on the second patient cohort receiving a higher dose of $2x10^{13}$ gc/kg, were presented at the 58th American Society of Hemophilia Annual Meeting.

  - Patients in the first dose cohort that discontinued prophylactic FIX infusions experienced an 85 percent reduction in total FIX usage and a marked reduction in frequency of spontaneous bleeding after treatment with AMT-060, including a complete cessation of spontaneous bleeding during the last 14 weeks of observation. The five patients in the second dose cohort experienced a near cessation of spontaneous bleeds with only one spontaneous bleed reported in 96 weeks of cumulative observation.

  - At both doses, AMT-060 appears to be safe and well-tolerated with no loss of FIX activity, no activation of T-cell response and no development of inhibitors for any of the 10 patients in the study. None of the 10 patients in the study tested positive for anti-AAV5 antibodies.

- **FDA Breakthrough Therapy Designation Granted for AMT-060 in Hemophilia B**

  - In January 2017, the U.S. Food and Drug Administration (FDA), granted Breakthrough Therapy Designation for AMT-060 in Hemophilia B patients. The designation is based on results from the ongoing Phase I/II study in patients with severe disease at up to 12 months follow-up. According to FDA data for its fiscal year 2016, the Center for Biologics Research and Review received a total of 23 requests for Breakthrough Therapy designation with only four designations granted, or 17% of all requests.

- **Initiated Regulatory Discussions for Phase III Program in Hemophilia B**

  - Regulatory discussions have now begun as the Company conducted its end-of-Phase II meeting with the FDA earlier this quarter. The meeting was positive, with the FDA acknowledging the clinical benefit of AMT-060 and expressing no safety concerns based on the Phase I/II data. The Company anticipates initiating discussions with the European Medicines Association later this year. Plans to advance the hemophilia B program into late-stage clinical development are ongoing, with a pivotal trial expected to begin in 2018.

- **Preclinical Data Published on AMT-130 in Huntington's Disease**

  - uniQure is conducting ongoing preclinical studies of AMT-130, its wholly-owned AAV5-based gene therapy product candidate for Huntington's disease. Preclinical data published in a peer-reviewed journal showed sustained and strong wild-type HTT protein silencing in humanized control mice, including knock-down efficiency up to 80% using optimized miHTT scaffolds. Data from additional preclinical studies will be presented during the year at various academic meetings. uniQure continues to advance AMT-130 towards filing an Investigational New Drug (IND) application to begin clinical studies in 2018.

- **Research Collaboration in Congestive Heart Failure Ongoing with Multiple Preclinical Studies**

  - Steady progress has been made with collaborator Bristol-Myers Squibb (BMS) in transferring S100A to an insect-cell preparation, manufacturing material for nonclinical and preclinical studies and conducting dose-ranging analyses and comparability studies in healthy and diseased pigs. This ongoing work is in support of an IND application expected to be filed by BMS in 2018.

- **Completion of a Company-wide Strategic Review**

  - uniQure announced the completion of a company-wide strategic review prioritizing programs in hemophilia B, Huntington's disease and cardiovascular disease, consolidating its GMP-manufacturing operations into its Lexington, MA location, streamlining its organization and strengthening its financial position.

- **Key Management and Board Appointments**

- uniQure strengthened its senior management team with the appointment of Paul Firuta as Chief Commercial Officer, Alex Kuta as Senior Vice President of Regulatory Affairs, Maria Cantor as Senior Vice President, Investor Relations & Communications, Jonathan Garen as Chief Business Officer and Maiken Keson-Brookes as Senior Vice President and General Counsel, all of whom are based in the U.S.

- Following its Annual General Meeting, the Company completed corporate governance changes in transitioning from a two-tier Supervisory Board and Management Board to a single Board of Directors structure with executive and non-executive members. In addition, the Company appointed Jack Kaye, a seasoned financial executive with more than 40 years of diversified experience, to its Board of Directors.

Matthew Kapusta was appointed by the Board of Directors as Chief Executive Officer (CEO). Mr. Kapusta had previously served as interim CEO and as Chief Financial Officer of the Company. Mr. Kapusta will continue to serve as an executive member of the uniQure Board of Directors.

Financial Highlights

Cash Position: As of December 31, 2016, the Company held cash and cash equivalents of $132.5 million, compared with $221.6 million as of December 31, 2015. The decrease in cash was primarily related to the advancement of its clinical and preclinical gene therapy targets, general corporate activities and capital expenditures related to its state-of-the-art manufacturing facility in Lexington, Massachusetts and the build-out of its new research facility in Amsterdam, the Netherlands. The Company intends to significantly reduce capital expenditures in 2017 and 2018 and realize operational cost savings from the strategic restructuring initiated in November 2016. As a result of these initiatives, the Company expects its cash on hand will be sufficient to fund operations into 2019.

Revenues: Revenue for 2016 was $25.1 million, compared with $10.6 million in 2015 and $6.1 million in 2014. The increases are driven by research activity associated with S100A1 for heart failure, which are fully reimbursed by BMS in accordance with the Company's collaboration agreement.

R&D Expenses: Research and development expenses were $72.5 million in 2016, compared with $59.1 million in 2015 and $43.8 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 intensification of the Company's activities to support the research of S100A1, the continued progression of uniQure's preclinical candidate for Huntington's disease, increased activity in the Company's U.S. facility and ongoing studies associated with the Company's collaboration with 4D Molecular Therapeutics to develop next-generation vector serotypes.

SG&A Expenses: Selling, general and administrative expenses were $26.0 million in 2016, compared with $23.4 million in 2015 and $17.1 million in 2014.

Other income, generated from research and development subsidies, was $1.5 million in 2016 compared with $0.8 million in 2015 and $1.0 million in 2014.

Net Loss: The net loss for the fourth quarter of 2016 was $14.7 million, or $0.58 per share, compared with $16.9 million, or $0.69 per share, for the fourth quarter of 2015. The net loss for the full years 2016, 2015 and 2014 was $73.4 million, or $2.93 per share, $82.1 million, or $3.72 per share, and $49.8 million, or $2.91 per share, respectively.

Form S-3 registration: In March 2015, the Company filed a "shelf registration statement" on Form F-3 with the Securities and Exchange Commission. Now that the Company reports as a domestic issuer, the F-3 registration statement is no longer effective, and therefore the Company today will file a new shelf registration statement on Form S-3. This will provide the Company with important financial flexibility for advancing its programs in the future. The Company has no immediate plans for any financing transaction.

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 liver/metabolic, central nervous system 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 implementation and effects of the Company's new strategic and organizational changes, the development of our gene therapy product candidates, the 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 corporate reorganizations and strategic shifts, 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 2015 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 4, 2016 and its 2016 Annual Report on Form 10-K filed 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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UNAUDITED CONSOLIDATED BALANCE SHEETS

	December 31, 2016	December 31, 2015

	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	$ 132,496	$ 221,626
Accounts receivable and accrued income	3,680	-
Accounts receivable and accrued income from related parties	5,500	4,129
Inventories	-	474
Prepaid expenses	996	690
Other current assets	1,274	1,194
Total current assets	**143,946**	**228,113**
Non-current assets		
Property, plant and equipment, net	35,702	26,011
Intangible assets, net	8,324	6,815
Goodwill	465	481
Other non-current assets	1,828	1,243
Total non-current assets	**46,319**	**34,550**
Total assets	**$ 190,265**	**$ 262,663**
Current liabilities		
Accounts payable	$ 5,524	$ 4,059
Accrued expenses and other current liabilities	9,766	9,863
Current portion of long-term debt	605	5,579
Current portion of deferred rent	684	630
Current portion of deferred revenue	6,142	6,778
Total current liabilities	**22,721**	**26,909**
Non-current liabilities		
Long-term debt, net of current portion	19,631	14,631
Deferred rent, net of current portion	6,781	6,247
Deferred revenue, net of current portion	75,612	83,445
Contingent consideration	1,838	2,926
Other non-current liabilities	51	578
Total non-current liabilities	**103,913**	**107,827**
Total liabilities	**126,634**	**134,736**
Commitments and contingencies		
Shareholders' equity		
Ordinary shares, €0.05 par value: 60,000,000 shares authorized at December 31, 2016 and 2015 and 25,257,420 and 24,327,944 shares issued and outstanding at December 31, 2016 and 2015, respectively.	1,593	1,542
Additional paid-in-capital	464,653	455,897
Accumulated other comprehensive loss	(6,557)	(6,828)
Accumulated deficit	(396,058)	(322,684)
Total shareholders' equity	**63,631**	**127,927**
Total liabilities and shareholders' equity	**$ 190,265**	**$ 262,663**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	Years ended December 31,		
	2016	**2015**	**2014**
	in thousands, except share and per share amounts		
Total revenues	**$ 25,098**	**10,578**	**6,141**
Operating expenses:			
Research and development expenses	(72,510)	(59,125)	(43,772)
Selling, general and administrative expenses	(25,999)	(23,383)	(17,073)

Total operating expenses	**(98,509)**	**(82,508)**	**(60,845)**
Other income	1,465	779	1,022
Loss from operations	**(71,946)**	**(71,151)**	**(53,682)**
Non operating items, net	**(283)**	**(12,111)**	**3,370**
Loss before income tax benefit / (expense)	**(72,229)**	**(83,262)**	**(50,312)**
Income tax benefit / (expense)	(1,145)	1,179	535
Net loss	**$ (73,374)**	**$ (82,083)**	**$ (49,777)**
Basic and diluted net loss per common share	$ (2.93)	$ (3.72)	$ (2.91)
Weighted average shares used in computing basic and diluted net loss per common share	25,036,465	22,082,345	17,121,328

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