

## uniQure Announces Financial Results For Third Quarter 2016 and Provides Update on Company Progress

*~ Strategic Review Completed to Refocus Pipeline, Reduce Operating Costs and Deliver Long-Term Shareholder Value*

*~ Updated Clinical Data on AMT-060 in Hemophilia B at Upcoming ASH Annual Meeting*

**Lexington, MA and Amsterdam, the Netherlands**, November 22, 2016 — uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced its financial results for the third quarter ended September 30, 2016 and provided an update on corporate developments.

“With the completion of our company-wide strategic review, we have focused our pipeline, streamlined operations and improved our financial position,” stated Matthew Kapusta, interim chief executive officer of uniQure. “Our near-term priorities are to aggressively prepare for a pivotal study in hemophilia B and to complete IND-enabling studies for our programs in Huntington’s disease and congestive heart failure. We look forward to the presentation of updated Phase I/II data on AMT-060 in a couple of weeks at ASH, and discussing our late-stage development plans.”

### Recent Highlights

- **Completed strategic review to refocus pipeline, simplify organization and reduce operating expenses** – On November 15, uniQure announced the completion of a company-wide strategic review aimed at focusing its pipeline, consolidating its manufacturing and enhancing overall execution to drive shareholder value. As a result of this initiative, the Company will prioritize programs in hemophilia B, Huntington’s disease and those associated with its landmark collaboration with Bristol-Myers Squib (BMS) in cardiovascular disease. Regarding the Company’s clinical product candidates in Sanfilippo B and Parkinsons’ disease, the Company has initiated discussions with its collaborators to explore options for these programs, including their potential transfer or partnership. Additionally, the Company will restructure its research and development organization in the Netherlands and consolidate manufacturing in the United States.
- **Updated Phase I/II data on AMT-060 at ASH; Preparations for pivotal study underway** – Professor Frank W.G. Leebeek, a clinical investigator at the Erasmus Medical Center in Rotterdam, the Netherlands, will be presenting new and updated data from uniQure’s ongoing Phase I/II clinical trial of AMT-060 at the 58<sup>th</sup> American Society of Hematology (ASH) Annual Meeting, on Saturday, December 3, 2016. The data will include up to 52 weeks of follow-up on the first patient cohort receiving  $5 \times 10^{12}$  gc/kg and up to 26 weeks of follow-up on the second patient cohort receiving a higher dose of  $2 \times 10^{13}$  gc/kg. In early 2017, uniQure expects to initiate interactions with regulatory authorities regarding a potential pivotal study of AMT-060 and will commence scaled-up production of clinical material in its Lexington, MA manufacturing facility.
- **Progressing IND-enabling studies of AMT-130** – uniQure is conducting ongoing preclinical studies of AMT-130, its wholly owned AAV5 gene therapy product candidate for Huntington’s disease. AMT-130 has demonstrated in preclinical studies up to 80 percent knockdown of the Huntingtin gene in the cortex and putamen in rat and humanized mouse models. Additional preclinical data, presentations and publications are expected in 2017 that will feature ongoing progress in moving toward filing an investigational new drug (IND) application to begin clinical studies.
- **Advancing S100A1 through multiple preclinical studies** – Significant progress has been made over the past year in transferring S100A1 from a mammalian system to an insect cell preparation, as well as conducting preclinical dose-ranging analyses and comparability studies. Various administration techniques have been analyzed and further animal studies are expected in 2017 to support an IND filing in 2018. uniQure also has begun early work on two additional cardiovascular targets, including vector development and other preclinical activities.

- **Maintaining strong cash position sufficient to fund operations into 2019** – As of September 30, 2016, the Company held cash and cash equivalents of €140.3 million. uniQure expects to realize significant cost savings as a result of the activities associated with its strategic restructuring. Specifically, uniQure expects to realize €5 million to €6 million of annualized cost savings in personnel and other related operating expenses as a result of the elimination of approximately 50 to 60 positions, or 20% to 25% of global headcount, by the end of 2017. Additionally, the Company expects to further reduce planned operating expenses by €11 million to €15 million over the next two years through the focusing of its pipeline. Based on its strong cash position and these cost savings, uniQure believes its existing cash resources will be sufficient to fund operations into 2019.

## Financial Highlights for Q3 2016

Revenues for the three months ended September 30, 2016 were €6.5 million, compared with €3.1 million for the comparable period in 2015. The increase relates to increased research activity associated with S100A1 for heart failure, the expenses of which are fully reimbursed by BMS in accordance with the Company's collaboration agreement.

Research and development expenses were €15.3 million for the three months ended September 30, 2016, compared with €11.9 million for the comparable period in 2015. The increase is mainly due to increased activity in support of our collaboration agreement with BMS, validation and scale-up activities related to AMT-060 in the Company's Lexington facility, increased facility costs in Amsterdam and the continued progression of our clinical program for AMT-060 in hemophilia B and preclinical candidate AMT-130 in Huntington's disease.

Selling, general and administrative expenses were €4.1 million for the three months ended September 30, 2016, compared with €4.8 million for the comparable period in 2015. The decrease results from a reduction in professional fees and a reversal of share based payment expenses for unvested equity grants to our former CEO upon his resignation in September 2016, offset in part by expenses associated with the Glybera Phase IV study and global registry, which in prior years were capitalized.

Losses related to foreign currency effects on U.S. dollar-denominated deposits were €0.6 million for the three months ended September 30, 2016, compared to €1.6 million for the comparable period in 2015. Net finance costs were €0.2 million for the three months ended September 30, 2016, compared to a gain of €1.2 million for the comparable period in 2015.

The net loss for the third quarter 2016 was €13.5 million, or €0.54 per share, compared with €25.8 million, or €1.08 per share, for the third quarter of 2015.

As of September 30, 2016, the Company held cash and cash equivalents of €140.3 million and had 25,143,760 ordinary shares outstanding. Cash used in operating and investing activities in the third quarter of 2016 includes approximately €6.6 million of one-time capital expenditures related to the refurbishment of a new R&D facility in Amsterdam, the Netherlands. Excluding these one-time expenditures related to the new facility, cash used in operating and investing activities in the third quarter of 2016 would have been €18.0 million.

## 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](http://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. 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.**  
**Unaudited Condensed Consolidated Statements of Financial Position**  
(€ in thousands)

	December 31, 2015	September 30, 2016
<b>Assets</b>		
<b>Non-current assets</b>		
Goodwill	442	442
Intangible assets other than Goodwill	7,209	8,771
Property, plant and equipment	23,820	29,730
Other non-current assets	1,142	1,663
Total non-current assets	32,613	40,606
<b>Current assets</b>		
Receivables from related parties	3,792	3,591
Trade and other receivables	1,730	6,142
Inventories	435	70
Cash and cash equivalents	203,532	140,331
Total current assets	209,489	150,134
<b>Total assets</b>	<b>242,102</b>	<b>190,740</b>
<b>Equity</b>		
Share capital	1,216	1,257
Share premium	344,803	346,753
Other reserves	26,026	30,361
Accumulated deficit	(252,561)	(305,989)
Total equity	119,484	72,382
<b>Liabilities</b>		
<b>Non-current liabilities</b>		
Borrowings	13,434	18,246
Derivative financial instruments - related party	530	98
Deferred rent	5,737	5,922
Deferred revenue	75,852	72,335
Contingent consideration	2,687	1,286
Total non-current liabilities	98,240	97,887
<b>Current liabilities</b>		
Trade and other payables	11,220	11,678
Derivative financial instruments - related parties	992	182
Borrowings	5,124	-
Borrowings - derivative	238	41
Deferred rent	579	597
Deferred revenue	6,225	5,573
Provisions	-	2,400
Total current liabilities	24,378	20,471
<b>Total liabilities</b>	<b>122,618</b>	<b>118,358</b>
<b>Total equity and liabilities</b>	<b>242,102</b>	<b>190,740</b>

**UNIQUE N.V.**  
**Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss**  
(€ in thousands except share and per share data)

	Three months ended September 30,		Nine months ended September 30,	
	2015	2016	2015	2016
License revenues	1,041	1,100	1,754	3,302
Collaboration revenues	1,809	5,366	3,818	11,007
Product sales	300	-	300	-
<b>Total revenues</b>	<b>3,150</b>	<b>6,466</b>	<b>5,872</b>	<b>14,309</b>
Cost of goods sold	(428)	(75)	(428)	(227)
Other income	186	302	532	1,126
Research and development expenses	(11,933)	(15,281)	(32,652)	(47,744)
Selling, general and administrative expenses	(4,835)	(4,140)	(13,503)	(16,248)
Impairment of intangible assets	(11,640)	-	(11,640)	-
Other gains/(losses), net	(1,552)	(623)	(2,548)	(5,002)
<b>Total operating costs</b>	<b>(30,202)</b>	<b>(19,817)</b>	<b>(60,239)</b>	<b>(68,095)</b>
<b>Operating loss</b>	<b>(27,052)</b>	<b>(13,351)</b>	<b>(54,367)</b>	<b>(53,786)</b>
Finance income	5	280	70	1,864
Finance expense	1,173	(450)	(3,102)	(1,506)
<b>Finance income/(expense), net</b>	<b>1,178</b>	<b>(170)</b>	<b>(3,032)</b>	<b>358</b>
<b>Result before corporate income tax</b>	<b>(25,874)</b>	<b>(13,521)</b>	<b>(57,399)</b>	<b>(53,428)</b>
Corporate income taxes	30	-	30	-
<b>Net loss</b>	<b>(25,844)</b>	<b>(13,521)</b>	<b>(57,369)</b>	<b>(53,428)</b>
Items that may be subsequently reclassified to profit or loss:				
Currency translation differences on foreign operations	(159)	(132)	927	(433)
Other comprehensive income/(loss)	(159)	(132)	927	(433)
<b>Total comprehensive loss</b>	<b>(26,003)</b>	<b>(13,653)</b>	<b>(56,442)</b>	<b>(53,861)</b>
Loss per share attributable to the equity holders of the Company during the period:				
<b>Basic and diluted loss per share</b>	<b>(1.08)</b>	<b>(0.54)</b>	<b>(2.69)</b>	<b>(2.14)</b>

**UNIQUE N.V.**  
**Unaudited Condensed Consolidated Statements of Changes in Equity/Deficit**  
(€ in thousands)

	Share capital	Share premium	Other reserves	Accumulated deficit	Total equity
<b>Balance at January 1, 2015</b>	<b>905</b>	<b>206,111</b>	<b>17,149</b>	<b>(181,081)</b>	<b>43,084</b>
Loss for the period	-	-	-	(57,369)	(57,369)
Other comprehensive income/(loss)	-	-	927	-	927
<b>Total comprehensive income/(loss)</b>	<b>-</b>	<b>-</b>	<b>927</b>	<b>(57,369)</b>	<b>(56,442)</b>
Capital contributions	306	138,877	-	-	139,183
Share issuance costs	-	(612)	-	-	(612)
Share based payment expense	-	-	5,242	-	5,242
<b>Balance at September 30, 2015</b>	<b>1,211</b>	<b>344,376</b>	<b>23,318</b>	<b>(238,450)</b>	<b>130,455</b>
Loss for the period	-	-	-	(14,111)	(14,111)
Other comprehensive income/(loss)	-	-	323	-	323
<b>Total comprehensive income/(loss)</b>	<b>-</b>	<b>-</b>	<b>323</b>	<b>(14,111)</b>	<b>(13,788)</b>
Capital contributions	5	427	-	-	432
Share based payment expense	-	-	2,385	-	2,385
<b>Balance at December 31, 2015</b>	<b>1,216</b>	<b>344,803</b>	<b>26,026</b>	<b>(252,561)</b>	<b>119,484</b>
Loss for the period	-	-	-	(53,428)	(53,428)
Other comprehensive income/(loss)	-	-	(433)	-	(433)
<b>Total comprehensive income/(loss)</b>	<b>-</b>	<b>-</b>	<b>(433)</b>	<b>(53,428)</b>	<b>(53,861)</b>
Capital contributions	41	1,950	-	-	1,991
Share based payment expense	-	-	4,768	-	4,768
<b>Balance at September 30, 2016</b>	<b>1,257</b>	<b>346,753</b>	<b>30,361</b>	<b>(305,989)</b>	<b>72,382</b>

**UNIQUE N.V.**  
**Unaudited Condensed Consolidated Statements of Cash Flows**  
(€ in thousands)

	Nine months ended September 30,	
	2015	2016
<b>Cash flow from operating activities</b>		
Net loss	(57,369)	(53,428)
Adjustments for:		
Amortization on intangible assets	274	600
Impairment of intangible assets	11,640	-
Depreciation	2,872	3,718
Lease incentive	129	(607)
Loss/(gain) on derivatives	164	(1,439)
Loss/(gain) on foreign exchanges	3,160	1,829
Share-based payment expenses	5,242	4,768
Changes in provisions	-	2,400
Changes in trade and other receivables	(3,339)	(4,211)
Changes in inventories	(210)	365
Changes in other current liabilities	1,933	429
Changes in deferred revenue	65,407	(4,169)
<b>Cash generated from/(used in) operations</b>	<b>29,903</b>	<b>(49,745)</b>
Interest paid	(1,404)	(1,434)
<b>Net cash generated from/(used in) operating activities</b>	<b>28,499</b>	<b>(51,179)</b>
<b>Cash used in investing activities</b>		
Purchases of property, plant and equipment	(3,289)	(9,502)
Purchases of intangible assets	(2,908)	(1,716)
Changes in restricted cash	-	(555)
Interest received	70	61
<b>Net cash used in investing activities</b>	<b>(6,127)</b>	<b>(11,712)</b>
<b>Cash flow from financing activities</b>		
Proceeds from shares issued	137,297	1,982
Payments of finance lease	(125)	(134)
<b>Net cash generated from financing activities</b>	<b>137,172</b>	<b>1,848</b>
<b>Net increase/(decrease) in cash and cash equivalents</b>	<b>159,544</b>	<b>(61,043)</b>
Currency effect cash and cash equivalents	1,731	(2,158)
Cash and cash equivalents at January 1	53,219	203,532
<b>Cash and cash equivalents at September 30</b>	<b>214,494</b>	<b>140,331</b>