



uniQure Announces Financial Results for Second Quarter 2015

Amsterdam, the Netherlands, August 27, 2015 - uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced financial results for the second quarter and six months ending June 30, 2015, and provided an update on multiple gene therapy programs.

"We are very pleased with the significant progress uniQure has made during the first half of 2015 and believe that uniQure is in a strong position to achieve its goal of delivering on the promise of gene therapy to patients," said Jörn Aldag, uniQure Chief Executive Officer. "The closing of our strategic collaboration with Bristol-Myers Squibb and our successful follow-on offering have substantially strengthened our financial position. We have also established three therapeutic focus areas with experienced leadership in place, and we welcome the addition of Charlie Richard to the uniQure team. As we move ahead in the second half of 2015, we look forward to the presentation of our first clinical data in hemophilia B and Sanfilippo B and to advancing our preclinical programs focused on CNS, liver/metabolic and cardiovascular diseases."

Pipeline Updates

- **Hemophilia B:** Dosing is underway in the Phase I/II study of AMT-060 in hemophilia B and the Company anticipates providing a preliminary readout of safety and efficacy data from the initial patients in the second half of 2015.
- **Sanfilippo B:** One-year follow-up results of the Phase I/II clinical trial conducted in Sanfilippo B patients are scheduled to be presented by the Institut Pasteur, uniQure's collaborator, at two scientific meetings in the second half of 2015. Details related to these presentations are provided at the end of this release.
- **Glybera®:** uniQure remains on target to initiate in early 2016 a U.S.-based clinical study for Glybera® (alipogen tiparvovec) to support post-approval requirements in the EU. In the Company's communications with the FDA regarding the U.S. regulatory pathway for Glybera, the FDA has stated they will require more than one additional clinical study to support a BLA filing. The Company is currently assessing its options for pursuing regulatory approval of Glybera in the U.S.
- **Parkinson's Disease:** The investigator-initiated Phase I clinical study of glial cell line-derived neurotrophic factor (GDNF) in Parkinson's disease, led by Krystof Bankiewicz, MD, PhD, at the University of California, San Francisco, has completed enrolment of its first dosing cohort and initiated dosing of its second cohort in the third quarter of 2015.

Corporate Highlights

Strategic Collaboration with Bristol-Myers Squibb

- On May 21, 2015, uniQure closed its collaboration agreement with Bristol-Myers Squibb to develop and commercialize gene therapies to treat a range of cardiovascular diseases and other therapeutic indications. In accordance with the collaboration agreement, uniQure received an upfront cash payment of \$50 million and issued 1.1 million ordinary shares to Bristol-Myers Squibb at \$33.84 per share for aggregate net proceeds of \$37.6 million.
- On August 10, 2015, uniQure announced the receipt of an additional \$53 million from Bristol-Myers Squibb in accordance with the companies' collaboration agreement. Included in the total was a \$15 million target designation fee triggered by Bristol-Myers Squibb's selection of three new collaboration targets, in addition to S100A1 for congestive heart failure. Bristol-Myers Squibb also acquired an additional 1.3 million ordinary shares of uniQure priced at \$29.67 per share, providing aggregate net proceeds to the Company of approximately \$38 million. The purchase price represented an approximately 26% premium over uniQure's closing price per ordinary share on August 7, 2015. After this second equity closing, Bristol-Myers Squibb owns 9.9% of uniQure's outstanding ordinary shares. To date, uniQure has received a total of \$140 million from Bristol-Myers Squibb in the context of the agreement.

Other Corporate Highlights

- **Follow-on Public Offering:** On April 15, 2015, uniQure completed a follow-on public offering of 3,000,000 ordinary shares at \$29.50 per ordinary share. After deducting the underwriting discounts and other offering expenses payable by uniQure, the aggregate net proceeds to the Company were approximately \$83.2 million.
- **Infrastructure:** uniQure's manufacturing site in Lexington, Massachusetts remains on target to achieve GMP readiness by the end of 2015.
- **Human Resources:** In an effort to drive greater patient focus and execution, uniQure has established three therapeutic focus areas in CNS, Liver/Metabolic and Cardiovascular disease indications. In July 2015, uniQure announced the appointment of Charles W. Richard, M.D., Ph.D., to the position of Senior Vice President, Research and Development, Neuroscience, to lead the Company's growing portfolio of gene therapies targeting neurological diseases, including current clinical trials for the treatment of Sanfilippo B syndrome and Parkinson's disease as well as preclinical programs in Huntington's disease and other rare CNS disorders.

uniQure is also pleased to announce the promotion of Deya Corzo, M.D., formerly Vice President, Medical Affairs at uniQure, to the position of Senior Vice President, Research and Development, Liver/Metabolic to lead the Company's development efforts in liver-directed and metabolic diseases, including hemophilia B, hemophilia A, and other rare liver/metabolic diseases. Together with Dr. Richard and Prof. Dr. Patrick Most, MD, Managing Director of uniQure GmbH and responsible for the Company's cardiovascular program, uniQure has significantly strengthened the leadership in its three therapeutic focus areas.

Financial Highlights

As of June 30, 2015, the Company held cash and cash equivalents of €181.9 million, compared with €53.2 million as of December 31, 2014. Licensing and collaboration revenues for the three months ended June 30, 2015 were €1.6 million, compared with €1.0 million for the comparable period in 2014. For the six months ended June 30, 2015, licensing and collaboration revenues were €2.7 million compared with €2.2 million for the same period of 2014. These revenues are related to the Company's collaboration agreements with Bristol-Myers Squibb and Chiesi.

Research and development expenses were €10.6 million for the three months ended June 30, 2015, compared with €8.0 million for the comparable period in 2014. For the six months ended June 30, 2015, research and development costs were €20.7 million compared with €14.2 million for the same period of 2014. The increase is related to the initiation of uniQure's Phase I/II clinical study of AMT-060 in hemophilia B, additional development and clinical activities required to support the planned commercial launch of Glybera, 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 €4.5 million for the three months ended June 30, 2015, compared with €2.5 million for the comparable period in 2014. Selling, general and administrative costs for the six months ended June 30, 2015 and 2014 were €8.7 million and €4.8 million, respectively. The increase was primarily due to expenses related to consultants and professional fees associated with business development and corporate finance matters and other general and administrative activities.

Other gains/losses were a loss of €5.2 million for the three months ended June 30, 2015, compared to a gain of €0.6 million for the comparable period in 2014. For the six months ended June 30, 2015, the gains/losses were a loss of €1.0 million compared with a gain of €0.06 million in the same period of 2014. The loss was primarily attributable to the timing of receipt of proceeds from the Company's follow-on public offering and the quarterly revaluation of outstanding warrants.

The net loss for the second quarter of 2015 was €18.9 million, or €0.87 per share, compared with €9.0 million, or €0.51 per share, for the second quarter of 2014. The net loss for the six months ended June 30, 2015 and 2014 were €31.5 million, or €1.58 per share and €16.8 million, or €1.03 per share, respectively.

For further financial information for the period ending June 30, 2015, please refer to the financial statements appearing at the end of this release.

Upcoming Sanfilippo B Data Presentations

*European Society of Gene and Cell Therapy (ESGCT) and Finnish Society of Gene Therapy (FSGT)
Collaborative Congress*

Location: Helsinki, Finland
Date: September 19, 2015
Session Time: 10:30am - 12:30pm

Abstract Title: Intra-cerebral administration of AAV vector containing the human alpha-N-acetylglucosaminidase cDNA in children with Sanfilippo type B (MPSIIIB) syndrome: results of a phase I/II trial

Speaker: Prof. M Tardieu

Sanfilippo Syndrome and Related Lysosomal Storage Diseases International Conference

Location: Geneva, Switzerland
Date: November 27, 2015
Session Time: 1:30pm - 3:00pm

Presentation Title: MPSIIB: Results at 12 months of a Phase I/II trial

Speaker: Prof. M Tardieu

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

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 our collaborations with BMS and other parties, the commercial launch of Glybera in the EU, the progress of any of the ongoing or planned clinical studies and/or development of our product candidates, and the commercialization or further regulatory approval of our products. 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, risks associated with our collaborations and collaboration partners, 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 7, 2015. 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 Balance Sheet
(€ in thousands)

| | DECEMBER 31, 2014 | JUNE 30, 2015 |
|--|--------------------------|----------------------|
| Assets | | |
| Non-current assets | | |
| Goodwill | 1,342 | 1,342 |
| Intangible assets other than Goodwill | 16,368 | 18,395 |
| Property, plant and equipment | 19,667 | 21,981 |
| Other non-current assets | 1,022 | 1,120 |
| Total non-current assets | 38,399 | 42,838 |
| Current assets | | |
| Receivables from related parties | 2,426 | 3,235 |
| Trade and other receivables | 1,542 | 2,097 |
| Inventories | 200 | 414 |
| Cash and cash equivalents | 53,219 | 181,855 |
| Total current assets | 57,387 | 187,601 |
| Total assets | 95,786 | 230,439 |
| Equity | | |
| Share capital | 905 | 1,136 |
| Share premium | 206,111 | 307,246 |
| Other reserves | 17,149 | 21,651 |
| Accumulated deficit | (181,081) | (212,606) |
| Total equity | 43,084 | 117,427 |
| Liabilities | | |
| Non-current liabilities | | |
| Borrowings | 16,418 | 15,092 |
| Derivative financial instruments - related parties | - | 1,137 |
| Financial lease liabilities | 134 | 45 |
| Deferred rent | 5,658 | 5,922 |
| Deferred revenue | 15,387 | 65,117 |
| Deferred tax liabilities | 1,379 | 1,379 |
| Contingent considerations | 1,454 | 1,966 |
| Total non-current liabilities | 40,430 | 90,658 |
| Current liabilities | | |
| Trade and other payables | 9,617 | 10,434 |
| Derivative financial instruments - related parties | 645 | 3,462 |
| Borrowings | - | 3,006 |
| Borrowings - derivative | 207 | 541 |
| Deferred rent | 475 | 544 |
| Deferred revenue | 1,328 | 4,367 |
| Total current liabilities | 12,272 | 22,354 |
| Total liabilities | 52,702 | 113,012 |
| Total equity and liabilities | 95,786 | 230,439 |

UNIQUE N.V.
Unaudited Condensed Consolidated Statements of Comprehensive Income

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

| | THREE MONTHS ENDED | | SIX MONTHS ENDED | |
|---|-----------------------------|-----------------|-------------------------|-----------------|
| | JUNE 30, | | JUNE 30, | |
| | 2014 | 2015 | 2014 | 2015 |
| | (€ in thousands) | | | |
| License revenues | 221 | 492 | 441 | 713 |
| Collaboration revenues | 821 | 1,123 | 1,771 | 2,009 |
| Total revenues | 1,042 | 1,615 | 2,212 | 2,722 |
| Other income | 152 | 140 | 390 | 346 |
| Research and development expenses | (8,008) | (10,613) | (14,226) | (20,719) |
| Selling, general and administrative expenses | (2,548) | (4,509) | (4,817) | (8,668) |
| Other gains / losses, net | 583 | (5,241) | 64 | (996) |
| Total operating costs | (9,821) | (20,223) | (18,589) | (30,037) |
| Operating result | (8,779) | (18,608) | (16,377) | (27,315) |
| Finance income | 44 | 46 | 71 | 65 |
| Finance expense | (255) | (325) | (514) | (4,275) |
| Finance income/(expense)--net | (211) | (279) | (443) | (4,210) |
| Result before corporate income tax | (8,990) | (18,887) | (16,820) | (31,525) |
| Corporate income taxes | - | - | - | - |
| Net loss | (8,990) | (18,887) | (16,820) | (31,525) |
| Items that may be subsequently reclassified to profit or loss | | | | |
| Currency translation differences on foreign operations | (11) | (285) | (10) | 1,086 |
| Other comprehensive income/(loss) | (11) | (285) | (10) | 1,086 |
| Total comprehensive loss | (9,001) | (19,172) | (16,830) | (30,439) |
| Loss per share attributable to the equity holders of the Company during the year: | | | | |
| Basic and diluted loss per share | (0.51) | (0.87) | (1.03) | (1.58) |

UNIQUE N.V.
Unaudited Condensed Consolidated Statement of Changes in Equity/Deficit

(€ in thousands)

| | Total | Share | Share | Other | Accumulated | Total |
|-------------------------------------|----------------|----------------|-----------------|------------------|-----------------------|--------------|
| | Capital | Premium | Reserves | Deficit | Equity/Deficit | |
| Balance at January 1, 2014 | 610 | 142,459 | 6,536 | (144,041) | 5,564 | |
| Result for the period | - | - | - | (16,820) | (16,820) | |
| Other comprehensive income/(loss) | - | - | - | (10) | (10) | |
| Total comprehensive loss | - | - | - | (16,830) | (16,830) | |
| Proceeds from shares issued | 270 | 62,351 | - | - | 62,621 | |
| Share issuance costs | - | (668) | - | - | (668) | |
| Share based payment/expense | - | - | 4,626 | - | 4,626 | |
| Balance at June 30, 2014 | 880 | 204,142 | 11,162 | (160,872) | 55,312 | |
| Result for the period | - | - | - | (20,218) | (20,218) | |
| Other comprehensive income | - | - | 1,149 | 9 | 1,158 | |
| Total comprehensive loss | - | - | 1,149 | (20,209) | (19,060) | |
| Capital contributions | 25 | 1,969 | - | - | 1,994 | |
| Share based payment/expense | - | - | 4,838 | - | 4,838 | |
| Balance at December 31, 2014 | 905 | 206,111 | 17,149 | (181,081) | 43,084 | |
| Result for the period | - | - | - | (31,525) | (31,525) | |
| Other comprehensive income | - | - | 1,086 | - | 1,086 | |
| Total comprehensive loss | - | - | 1,086 | (31,525) | (30,439) | |
| Capital contributions | 231 | 101,747 | - | - | 101,978 | |
| Share issuance costs | - | (612) | - | - | (612) | |
| Share based payment/expense | - | - | 3,416 | - | 3,416 | |
| Balance at June 30, 2015 | 1,136 | 307,246 | 21,651 | (212,606) | 117,427 | |

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

| | SIX MONTHS ENDED | |
|--|-------------------------|----------------|
| | June 30, | |
| | 2014 | 2015 |
| Cash flow from operating activities | | |
| Net loss | (16,820) | (31,525) |
| Adjustments for: | | |
| Depreciation | 310 | 1,877 |
| Lease incentive | 3,876 | 333 |
| Loss/(gain) on derivatives | (253) | 1,666 |
| Loss/(gain) on foreign exchanges | (64) | 1,873 |
| Other non-cash items | (9) | 511 |
| Share-based expenses | 4,626 | 3,416 |
| Changes in trade and other receivables | (292) | (1,363) |
| Movement in inventories | 438 | (214) |
| Changes in trade and other payables | (1,240) | (5,922) |
| Changes in deferred revenue and provisions | (377) | 52,769 |
| Initial recognition of warrants | - | 2,622 |
| Movement in other liabilities | 448 | 1,581 |
| Interest (income) / expense | 650 | 976 |
| Cash (used in) / generated by operations | (8,707) | 28,600 |
| Interest paid | (461) | (929) |
| Net cash (used in) / generated by operating activities | (9,168) | 27,671 |
| Cash flow from investing activities | | |
| Purchases of property, plant and equipment | (9,787) | (1,855) |
| Purchases of intangible assets | (1,953) | (1,729) |
| Interest received | 59 | 59 |
| Net cash used in investing activities | (11,681) | (3,525) |
| Cash flow from financing activities | | |
| Proceeds from shares issued | 62,621 | 101,978 |
| Share issuance cost | (668) | (612) |
| Exchange result on Borrowings | 46 | - |
| Proceeds from Borrowings | 7,184 | - |
| Payments of finance lease | (77) | (82) |
| Net cash generated from financing activities | 69,106 | 101,284 |
| Net increase in cash, cash equivalents and bank overdrafts | 48,257 | 125,430 |
| Currency effect cash and cash equivalents | (10) | 3,206 |
| Cash, cash equivalents and bank overdrafts at beginning of the period | 23,810 | 53,219 |
| Cash, cash equivalents and bank overdrafts at end of the period | 72,057 | 181,855 |