# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

# FORM 6-K

Report of Foreign Private Issuer Pursuant to Rule 13a-16 or 15d-16 of the Securities Exchange Act of 1934

June 11, 2015

# uniQure N.V.

Jörn Aldag, Chief Executive Officer Meibergdreef 61 Amsterdam 1105 BA, the Netherlands; Tel: +31 20 566 7394 (Address, Including ZIP Code, and Telephone Number, Including Area Code, of Registrant's Principal Executive Offices)

Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F.

Form 20-F x Form 40-F o

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1): o

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7): o

Furnished as Exhibit 99.1 to this Report on Form 6-K is a press release of uniQure N.V. dated June 11, 2015, announcing the Company's unaudited financial results for the first quarter ended March 31, 2015; and furnished as Exhibit 99.2 to this Report on Form 6-K are the Company's unaudited financial statements for the first quarter ended March 31, 2015.

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## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

UNIQURE N.V.

Date: June 11, 2015

By: <u>/S/ JÖRN ALDAG</u> Jörn Aldag Chief Executive Officer

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## INDEX TO EXHIBITS

| Number | Description   |
|--------|---|
| 99.1   | Press release of uniQure N.V. dated June 11, 2015, announcing the Company's unaudited financial results for the first quarter ended |
| 33.1   | March 31, 2015.   |
| 99.2   | Unaudited financial statements for the first quarter ended March 31, 2015.  |



#### FOR IMMEDIATE RELEASE

### uniQure Announces Financial Results for First Quarter 2015

**Amsterdam, the Netherlands, June 11, 2015** — uniQure N.V. (NASDAQ: QURE), a leader in human gene therapy, today announced financial results for the first quarter ending March 31, 2015, and provided an update on multiple gene therapy programs.

## **Pipeline Updates**

- **Hemophilia B:** The Company has commenced screening patients in its first clinical trial site in Germany for its Phase I/II study of AMT-060 in hemophilia B patients and anticipates providing a preliminary readout of safety and efficacy data in the second half of 2015.
- **Sanfilippo B:** All patients have been dosed in the collaborator-sponsored Sanfilippo B program with Institut Pasteur. One year follow-up results will be available in the second half of 2015 and presented at a relevant scientific meeting.
- **Glybera**<sup>®</sup>: uniQure remains on target to initiate in early 2016 a pivotal clinical study for Glybera<sup>®</sup> (alipogen tiparvovec) to support a future regulatory submission in the U.S.
- Parkinson's Disease: uniQure's investigator-sponsored 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 will be commencing dosing of its second cohort in the third quarter of 2015.

## **Corporate Highlights**

#### Strategic Collaboration with Bristol-Myers Squibb

- On May 26, 2015, uniQure announced the closing of its collaboration with Bristol-Myers Squibb Company, triggering an initial \$50 million upfront payment. The partnership, announced April 6, 2015, provides BMS with exclusive access to uniQure's gene therapy technology platform for multiple targets in the cardiovascular space. The collaboration includes uniQure's proprietary congestive heart failure program targeting S100A1, a calcium sensor and master regulator of heart function, and the companies will collaborate on up to nine other gene therapy targets addressing a broad range of cardiovascular disease and other target-specific disease areas.
- On June 10, 2015, the shareholders of uniQure approved the equity component of the BMS collaboration, and the Company will issue to BMS an initial tranche of approximately 1.1 million ordinary shares, at a purchase price of \$33.84 per share, for aggregate consideration of approximately \$38 million. The transaction is expected to close on June 12, 2015.

#### **Business Development**

- Collaboration with Treeway: In January 2015, uniQure entered into a license and collaboration agreement with Treeway B.V., a private company founded by entrepreneurs Bernard Muller and Robbert Jan Stuit, both diagnosed with amyotrophic lateral sclerosis, or ALS, to develop a gene therapy treatment for ALS.
- Collaboration with Synpromics: In January 2015, uniQure entered into a collaborative license agreement with Synpromics Limited to strengthen its technology platform with respect to therapeutic indications that require high-level therapeutic gene expression or comprise large therapeutic genes. uniQure will exclusively own the results of this collaborative effort.

## 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 estimated offering expenses payable by uniQure, the net proceeds to the Company were approximately \$82.5 million (€77.9 million).
- **Infrastructure**: In the first quarter of 2015, the Company completed the build-out of its 53,000 sq. ft. manufacturing facility in Lexington, Massachusetts and remains on target to achieve GMP readiness by the end of 2015.
- Human Resources: In January 2015, uniQure announced the appointment of Matt Kapusta to Chief Financial Officer. On June 10, the shareholders of uniQure approved the appointments of Mr. Kapusta to the Management Board and Philip Astley-Sparke to the Supervisory Board. In addition, uniQure leadership accepted the request by Chief Commercial Officer Hans Christian Rohde to proceed with early retirement effective August 1, 2015.

"The completion of our landmark collaboration with Bristol-Myers Squibb begins an exciting new chapter for uniQure by advancing our goal to bring the promise of gene therapy to the millions of patients with cardiovascular disease," Jörn Aldag, uniQure Chief Executive Officer, commentated. "In conjunction with our successful follow-on offering, we also now have the necessary financial resources to achieve proof-of-concept in hemophilia B, Sanfilippo B and

Parkinson's disease and further the clinical development of these important programs. We also plan to expand our proprietary pipeline in rare liver/ metabolic and CNS diseases by advancing several preclinical product candidates and pursuing acquisitions and in-licensing opportunities."

Mr. Aldag added: "All of us at uniQure would like to thank Hans Christian for his leadership and collaboration over the last three years and we wish him the best for his well-deserved retirement."

### **Financial Highlights**

As of March 31, 2015, the Company held cash and cash equivalents of  $\notin$ 43.2 million, compared with  $\notin$ 53.2 million as of December 31, 2014. Licensing and collaboration revenues for the three months ended March 31, 2015 were  $\notin$ 1.1 million, compared with  $\notin$ 1.2 million for the comparable period in 2014. The majority of 2015 revenues are related to development activities that were reimbursable by Chiesi under the Company's co-development agreement for hemophilia B.

Research and development expenses were  $\leq 10.1$  million for the three months ended March 31, 2015, compared with  $\leq 6.2$  million for the comparable period in 2014. The increase is related to the initiation of our Phase I/II clinical study of AMT-060 in hemophilia, 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 our U.S. facility.

Selling, general and administrative expenses were &4.2 million for the three months ended March 31, 2015, compared with &2.3 million or the comparable period in 2014. The increase is primarily due to expenses related to consultants and professional fees, associated with business development and other general and administrative activities. The net loss for the first quarter 2015 was &12.6 million, or &0.69 per share, compared with &7.8 million, or &0.52 per share, for the first three months of 2014.

### 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 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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### UNIQURE N.V. Unaudited Condensed Consolidated Balance Sheet (€ in thousands)

|                                       | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|---------------------------------------|----------------------|-------------------|
| Assets                                |                      |                   |
| Non-current assets                    |                      |                   |
| Goodwill                              | 1,342                | 1,342             |
| Intangible assets other than Goodwill | 16,368               | 17,473            |
| Property, plant and equipment         | 19,667               | 22,439            |
| Other non-current assets              | 1,022                | 1,145             |

| Total non-current assets           | 38,399    | 42,399    |
|------------------------------------|-----------|-----------|
| Current assets                     |           |           |
| Receivables from related parties   | 2,426     | 2,445     |
| Trade and other receivables        | 1,542     | 1,935     |
| Inventories                        | 200       | 154       |
| Cash and cash equivalents          | 53,219    | 43,197    |
| Total current assets               | 57,387    | 47,731    |
| Total assets                       | 95,786    | 90,130    |
| Equity                             |           |           |
| Share capital                      | 905       | 921       |
| Share premium                      | 206,111   | 206,242   |
| Other reserves                     | 17,149    | 20,329    |
| Accumulated deficit                | (181,081) | (193,719) |
| Total equity                       | 43,084    | 33,773    |
| Liabilities                        |           |           |
| Non-current liabilities            |           |           |
| Borrowings                         | 16,418    | 17,050    |
| Financial lease liabilities        | 134       | 90        |
| Deferred rent                      | 5,658     | 6,201     |
| Deferred revenue                   | 15,387    | 15,237    |
| Deferred tax liabilities           | 1,379     | 1,379     |
| Contingent considerations          | 1,454     | 1,251     |
| Total non-current liabilities      | 40,430    | 41,208    |
| Current liabilities                |           |           |
| Trade and other payables           | 9,617     | 9,345     |
| Debt to related party - derivative | 645       | 1,829     |
| Borrowings                         | —         | 1,402     |
| Borrowings - derivative            | 207       | 490       |
| Deferred rent                      | 475       | 544       |
| Deferred revenue                   | 1,328     | 1,539     |
| Total current liabilities          | 12,272    | 15,149    |
| Total liabilities                  | 52,702    | 56,357    |
| Total equity and liabilities       | 95,786    | 90,130    |

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## UNIQURE N.V.

## Unaudited Condensed Consolidated Statements of Comprehensive Income

( $\in$  in thousands, except share and per share data)

|   | THREE MONTHS ENDED<br>MARC H 31, |          |
|---|----------------------------------|----------|
|   | 2014                             | 2015     |
|   | (€ in thousands)                 |          |
| License revenues  | 220                              | 221      |
| Collaboration revenues  | 950                              | 886      |
| Total revenues  | 1,170                            | 1,107    |
| Cost of goods sold  | —                                | —        |
| Other income  | 238                              | 206      |
| Research and development expenses   | (6,218)                          | (10,106) |
| Selling, general and administrative expenses                                      | (2,268)                          | (4,159)  |
| Other gains / losses, net   | (519)                            | 4,245    |
| Total operating costs   | (8,767)                          | (9,814)  |
| Operating result  | (7,597)                          | (8,707)  |
| Finance income  | 27                               | 19       |
| Finance expense   | (259)                            | (3,950)  |
| Finance income/(expense)—net  | (232)                            | (3,931)  |
| Result before corporate income tax  | (7,829)                          | (12,638) |
| Corporate income taxes  | —                                |          |
| Net loss  | (7,829)                          | (12,638) |
| Items that may be subsequently reclassified to profit or loss                     | 2                                | 1,371    |
| Other comprehensive income  | 2                                | 1,371    |
| Total comprehensive loss  | (7,827)                          | (11,267) |
| Loss per share attributable to the equity holders of the Company during the year: |                                  |          |
| Basic and diluted loss per share  | (0.52)                           | (0.69)   |
|   |                                  |          |

## UNIQURE N.V.

|                              | Total<br>Share<br>Capital | Share<br>Premium | Other<br>Reserves | Accumulated<br>Deficit | Total<br>Equity/Deficit |
|------------------------------|---------------------------|------------------|-------------------|------------------------|-------------------------|
| Balance at January 1, 2014   | 610                       | 142,459          | 6,536             | (144,041)              | 5,564                   |
| Result for the period        | —                         | —                |                   | (7,829)                | (7,829)                 |
| Other comprehensive income   | —                         | —                | —                 | 1                      | 1                       |
| Total comprehensive loss     |                           |                  |                   | (7,828)                | (7,828)                 |
| Proceeds from shares issued  | 270                       | 62,351           |                   |                        | 62,621                  |
| Share issuance costs         | —                         | (668)            |                   | —                      | (668)                   |
| Share based payment/expense  | —                         | —                | 2,342             | —                      | 2,342                   |
| Balance at March 31, 2014    | 880                       | 204,142          | 8,878             | (151,870)              | 62,030                  |
| Result for the period        |                           |                  |                   | (29,209)               | (29,209)                |
| Other comprehensive income   | —                         | —                | 1,149             | (2)                    | 1,147                   |
| Total comprehensive loss     |                           |                  | 1,149             | (29,211)               | (28,062)                |
| Capital contributions        | 25                        | 1,969            |                   |                        | 1,994                   |
| Share based payment/expense  |                           | —                | 7,122             | —                      | 7,122                   |
| Balance at December 31, 2014 | 905                       | 206,111          | 17,149            | (181,081)              | 43,084                  |
| Result for the period        |                           |                  |                   | (12,638)               | (12,638)                |
| Other comprehensive income   | _                         |                  | 1,371             |                        | 1,371                   |
| Total comprehensive loss     |                           | _                | 1,371             | (12,638)               | (11,267)                |
| Capital contributions        | 16                        | 131              |                   |                        | 147                     |
| Share based payment/expense  |                           | —                | 1,809             | —                      | 1,809                   |
| Balance at March 31, 2015    | 921                       | 206,242          | 20,329            | (193,719)              | 33,773                  |

## UNIQURE N.V.

## Unaudited Condensed Consolidated Statement of Cash Flows

## (€ in thousands)

|   | THREE MONTHS<br>March 31, |          |
|---|---------------------------|----------|
|   | 2014                      | 2015     |
| Cash flow from operating activities                                   |                           |          |
| Net loss  | (7,829)                   | (12,638) |
| Adjustments for:  |                           |          |
| Depreciation  | 144                       | 897      |
| Lease incentive   | 2,854                     | 613      |
| Derivative result   | (10)                      | 1,467    |
| Exchange result   | 7                         | (3,044)  |
| Other non-cash items  | —                         | (203)    |
| Share-based expenses  | 2,342                     | 1,809    |
| Changes in trade and other receivables                                | (320)                     | (411)    |
| Movement in inventories   | (83)                      | 47       |
| Changes in trade and other payables                                   | (1,062)                   | (1,580)  |
| Changes in deferred revenue and provisions                            | (213)                     | 61       |
| Movement in other liabilities   | (909)                     | 896      |
| Interest (income) / expense   | 235                       | 490      |
| Cash used in operations   | (4,844)                   | (11,596) |
| Interest paid   | (225)                     | (449)    |
| Net cash used in operating activities                                 | (5,069)                   | (12,045) |
| Cash flow from investing activities                                   |                           |          |
| Purchases of property, plant and equipment                            | (2,025)                   | (1,268)  |
| Purchases of intangible assets  | (1,148)                   | (769)    |
| Interest received   | 47                        | 30       |
| Net cash used in investing activities                                 | (3,126)                   | (2,007)  |
| Cash flow from financing activities                                   | iii                       | i        |
| Proceeds from shares issued   | 62,621                    | 147      |
| Share issuance cost   | (668)                     |          |
| Redemption of financial lease   | (38)                      | (41)     |
| Net cash generated from financing activities                          | 61,915                    | 106      |
| Net increase in cash, cash equivalents and bank overdrafts            | 53,720                    | (13,946) |
| Currency effect cash and cash equivalents                             | 2                         | 3,924    |
| 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       | 77,532                    | 43,197   |
| -   | /                         |          |

## UNIQURE N.V.

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## UNIQURE N.V.

## **Unaudited Condensed Consolidated Balance Sheets**

(€ in thousands)

|                                       | NOTE  | DECEMBER 31,<br>2014                  | MARCH, 31<br>2015 |
|---------------------------------------|-------|---------------------------------------|-------------------|
| Assets                                |       |                                       |                   |
| Non-current assets                    |       |                                       |                   |
| Goodwill                              | 8,9   | 1,342                                 | 1,342             |
| Intangible assets other than Goodwill | 8,9   | 16,368                                | 17,473            |
| Property, plant and equipment         | 7     | 19,667                                | 22,439            |
| Other non-current assets              | 10    | 1,022                                 | 1,145             |
| Total non-current assets              |       | 38,399                                | 42,399            |
| Current assets                        |       |                                       |                   |
| Receivables from related parties      | 11    | 2,426                                 | 2,445             |
| Trade and other receivables           | 11    | 1,542                                 | 1,935             |
| Inventories                           | 12    | 200                                   | 154               |
| Cash and cash equivalents             | 13    | 53,219                                | 43,197            |
| Total current assets                  |       | 57,387                                | 47,731            |
| Total assets                          |       | 95,786                                | 90,130            |
| Equity                                |       |                                       |                   |
| Share capital                         |       | 905                                   | 921               |
| Share premium                         |       | 206,111                               | 206,242           |
| Other reserves                        |       | 17,149                                | 20,329            |
| Accumulated deficit                   |       | (181,081)                             | (193,719)         |
| Total equity                          | 14    | 43,084                                | 33,773            |
| Liabilities                           |       |                                       |                   |
| Non-current liabilities               |       |                                       |                   |
| Borrowings                            | 16    | 16,418                                | 17,050            |
| Financial lease liabilities           | 16,26 | 134                                   | 90                |
| Deferred rent                         | 24    | 5,658                                 | 6,201             |
| Deferred revenue                      | 17    | 15,387                                | 15,237            |
| Deferred tax liabilities              | 9     | 1,379                                 | 1,379             |
| Contingent considerations             | 9     | 1,454                                 | 1,251             |
| Total non-current liabilities         |       | 40,430                                | 41,208            |
| Current liabilities                   |       | · · · · · · · · · · · · · · · · · · · |                   |
| Trade and other payables              | 15    | 9,617                                 | 9,345             |
| Debt to related party - derivative    | 16    | 645                                   | 1,829             |
| Borrowings                            | 16    | _                                     | 1,402             |
| Borrowings - derivative               | 16    | 207                                   | 490               |
| Deferred rent                         | 26    | 475                                   | 544               |
| Deferred revenue                      | 17    | 1,328                                 | 1,539             |
| Total current liabilities             |       | 12,272                                | 15,149            |
| Total liabilities                     |       | 52,702                                | 56,357            |
| Total equity and liabilities          |       | 95,786                                | 90,130            |
| • •                                   |       |                                       |                   |

The notes are an integral part of these Condensed Consolidated Financial Statements.

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|   |      | MARCH 31, |          | MARCH 31, | 1, |
|---|------|-----------|----------|-----------|----|
|   | NOTE | 2014      | 2015     |           |    |
| License revenues  | 17   | 220       | 221      |           |    |
| Collaboration revenues  | 17   | 950       | 886      |           |    |
| Total revenues  |      | 1,170     | 1,107    |           |    |
| Cost of goods sold  |      | —         | _        |           |    |
| Other income  |      | 238       | 206      |           |    |
| Research and development expenses                             | 18   | (6,218)   | (10,106) |           |    |
| Selling, general and administrative expenses                  | 19   | (2,268)   | (4,159)  |           |    |
| Other gains / losses, net                                     | 20   | (519)     | 4,245    |           |    |
| Total operating costs   |      | (8,767)   | (9,814)  |           |    |
| Operating result  |      | (7,597)   | (8,707)  |           |    |
| Finance income  |      | 27        | 19       |           |    |
| Finance expense   | 22   | (259)     | (3,950)  |           |    |
| Finance income/(expense)—net                                  |      | (232)     | (3,931)  |           |    |
| Result before corporate income tax                            |      | (7,829)   | (12,638) |           |    |
| Corporate income taxes  |      |           | —        |           |    |
| Net loss  |      | (7,829)   | (12,638) |           |    |
| Items that may be subsequently reclassified to profit or loss |      | 2         | 1,371    |           |    |
| Other comprehensive income                                    | 21   | 2         | 1,371    |           |    |
| Total comprehensive loss                                      |      | (7,827)   | (11,267) |           |    |
| Loss per share attributable to the equity holders of the      |      |           |          |           |    |
| Company during the year:                                      |      |           |          |           |    |
| Basic and diluted loss per share                              | 24   | (0.52)    | (0.69)   |           |    |

The notes are an integral part of these Condensed Consolidated Financial Statements.

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## UNIQURE N.V.

## Unaudited Condensed Consolidated Statement of Changes in Equity/Deficit

(€ in thousands)

|                              | Note | Total<br>Share<br>Capital | Share<br>Premium | Other<br>Reserves | Accumulated<br>Deficit | Total<br>Equity/Deficit |
|------------------------------|------|---------------------------|------------------|-------------------|------------------------|-------------------------|
| Balance at January 1, 2014   |      | 610                       | 142,459          | 6,536             | (144,041)              | 5,564                   |
| Result for the period        |      |                           | —                | —                 | (7,829)                | (7,829)                 |
| Other comprehensive income   |      |                           |                  |                   | 2                      | 2                       |
| Total comprehensive loss     |      |                           |                  | _                 | (7,827)                | (7,827)                 |
| Proceeds from shares issued  |      | 270                       | 62,351           |                   |                        | 62,621                  |
| Share issuance costs         |      |                           | (668)            |                   |                        | (668)                   |
| Share based payment/expense  |      | —                         | —                | 2,342             | —                      | 2,342                   |
| Balance at March 31, 2014    |      | 880                       | 204,142          | 8,878             | (151,869)              | 62,031                  |
| Result for the period        |      |                           |                  |                   | (29,210)               | (29,210)                |
| Other comprehensive income   |      | —                         | _                | 1,149             | (2)                    | 1,147                   |
| Total comprehensive loss     |      |                           | _                | 1,149             | (29,212)               | (28,063)                |
| Capital contributions        |      | 25                        | 1,969            |                   |                        | 1,994                   |
| Share based payment/expense  |      |                           |                  | 7,122             | —                      | 7,122                   |
| Balance at December 31, 2014 | 14   | 905                       | 206,111          | 17,149            | (181,081)              | 43,084                  |
| Result for the period        |      |                           |                  |                   | (12,638)               | (12,638)                |
| Other comprehensive income   |      | _                         | _                | 1,371             | _                      | 1,371                   |
| Total comprehensive loss     |      |                           | _                | 1,371             | (12,638)               | (11,267)                |
| Capital contributions        |      | 16                        | 131              |                   |                        | 147                     |
| Share based payment/expense  |      | —                         | —                | 1,809             | —                      | 1,809                   |
| Balance at March 31, 2015    | 14   | 921                       | 206,242          | 20,329            | (193,719)              | 33,773                  |

The notes are an integral part of these Condensed Consolidated Financial Statements.

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## UNIQURE N.V.

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

|                                     |      | THREE MONTHS ENDED<br>March 31, |          |  |
|-------------------------------------|------|---------------------------------|----------|--|
|                                     | NOTE | 2014                            | 2015     |  |
| Cash flow from operating activities |      |                                 |          |  |
| Net loss                            |      | (7,829)                         | (12,638) |  |
|                                     |      |                                 |          |  |

| Adjustments for:  |    |         |          |
|---|----|---------|----------|
| Depreciation  | 7  | 144     | 897      |
| Lease incentive   | 26 | 2,854   | 613      |
| Derivative result   | 16 | (10)    | 1,467    |
| Exchange result   |    | 7       | (3,044)  |
| Other non-cash items  |    | —       | (203)    |
| Share-based expenses  | 23 | 2,342   | 1,809    |
| Changes in trade and other receivables                                |    | (320)   | (411)    |
| Movement in inventories   | 12 | (83)    | 47       |
| Changes in trade and other payables                                   | 15 | (1,062) | (1,580)  |
| Changes in deferred revenue and provisions                            |    | (213)   | 61       |
| Movement in other liabilities   |    | (909)   | 896      |
| Interest (income) / expense   |    | 235     | 490      |
| Cash used in operations   |    | (4,844) | (11,596) |
| Interest paid   |    | (225)   | (449)    |
| Net cash used in operating activities                                 | -  | (5,069) | (12,045) |
| Cash flow from investing activities                                   | _  |         |          |
| Purchases of property, plant and equipment                            | 7  | (2,025) | (1,268)  |
| Purchases of intangible assets  | 8  | (1,148) | (769)    |
| Interest received   |    | 47      | 30       |
| Net cash used in investing activities                                 | _  | (3,126) | (2,007)  |
| Cash flow from financing activities                                   | _  |         |          |
| Proceeds from shares issued   | 14 | 62,621  | 147      |
| Share issuance cost   | 14 | (668)   | _        |
| Redemption of financial lease   | 16 | (38)    | (41)     |
| Net cash generated from financing activities                          | -  | 61,915  | 106      |
| Net increase in cash, cash equivalents and bank overdrafts            | -  | 53,720  | (13,946) |
| Currency effect cash and cash equivalents                             | _  | 2       | 3,924    |
| 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       |    | 77,532  | 43,197   |
| - •   | =  |         | <u>,</u> |

The notes are an integral part of these Condensed Consolidated Financial Statements.

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## UNIQURE N.V.

## Notes to Unaudited Condensed Consolidated Financial Statements

## 1. General information

## uniQure N.V.

uniQure N.V. ("uniQure" or the "Company") is a biopharmaceutical company domiciled in The Netherlands with headquarters at Meibergdreef 61, 1105 BA, Amsterdam, The Netherlands.

The Company is a leader in the field of gene therapy, and has developed the first product to receive regulatory approval in the European Union and has multiple collaborations designed to accelerate the development of a broad pipeline of additional product candidates. The Company was incorporated in January 2012 to acquire and continue the gene therapy business ("AMT Business") of Amsterdam Molecular Therapeutics (AMT) Holding N.V. ("AMT") and its subsidiaries (collectively, the "AMT Group") and to facilitate additional financing, as described further below. As used in these Condensed Consolidated Interim Financial Statements, unless the context indicates otherwise, all references to "uniQure" or the "Company" refer to uniQure and its Consolidated Subsidiaries.

### Organizational structure of the uniQure Group

uniQure N.V. is the ultimate parent of the following group of entities:

| Company name                     |
|----------------------------------|
| uniQure biopharma B.V.           |
| uniQure IP B.V.                  |
| uniQure Manufacturing B.V.       |
| uniQure Assay Development B.V.   |
| uniQure Research B.V.            |
| uniQure non clinical B.V.        |
| uniQure QA B.V.                  |
| uniQure Process Development B.V. |
| uniQure clinical B.V.            |
| Stichting participatie AMT(1)    |
| uniQure Inc.                     |
| uniQure GmbH (2)                 |

- (1) Stichting participatie AMT is a Trust, not a company, but met the conditions for consolidation within uniQure's Consolidated Financial Statements. Stichting participatie AMT was established to facilitate AMT's employee incentive schemes for the period up to 2010.
- (2) In July 2014 the company acquired InoCard GmbH, renamed to uniQure GmbH in August 2014.

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#### **Other matters**

On January 20, 2014, the shareholders of the Company approved, and on January 21, 2014 the supervisory board of the Company confirmed, a 5-for-1 consolidation of shares, which had the effect of a reverse share split, that became effective on January 31, 2014. All share, per-share and related information presented in these unaudited Condensed Consolidated Financial Statements and accompanying footnotes has been retroactively adjusted, where applicable, to reflect the impact of the reverse share split.

On February 5, 2014 the Company successfully completed its initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91,800,000 ( $\in$ 67,300,000) and net proceeds of \$85,400,000 ( $\in$ 62,621,000) after commissions but before expenses. At the time of the initial public offering all existing shareholders agreed to a 180 day lock-up that has expired on August 4, 2014.

On July 15, 2014 the Company signed and on July 31, 2014 the Company closed an agreement to acquire all shares of InoCard GmbH. For further disclosures please refer to note 9.

On April 6, 2015, the Company entered into agreements with BMS, which provide BMS exclusive access to uniQure's gene therapy technology platform for multiple targets in cardiovascular and other target-specific disease areas. The collaboration includes the Company's proprietary congestive heart failure gene therapy program, which has demonstrated in advanced preclinical models that it can restore the ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and increase survival rates after myocardial infarction. In addition, the Company will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of cardiovascular and other target-specific disease areas. uniQure will be responsible for discovery, preclinical development, and CMC, and will provide BMS its vector technologies and access to its industrial, proprietary insect-cell based manufacturing platform. uniQure will be responsible for CMC portions of regulatory filings, and will co-operate with BMS in the preparation of all regulatory materials and interactions with regulatory authorities. BMS will be responsible for clinical development and all commercial activities across all programs.

The financial terms consist of guaranteed, near-term payments to uniQure of \$103 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the transaction (other than the initial equity investment) occurred on May 21, 2015. An additional \$15 million payment is to be received following the selection of three additional collaboration targets, in addition to the S100A1 program, within three months of the closing. In addition, an initial equity investment in uniQure will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or about \$38 million in total. This investment is expected to be completed in the second quarter of 2015. BMS is also obligated to make an additional equity investment in uniQure for a number of shares that will equal 5.0% of the total number of shares outstanding following such issuance by December 31, 2015 and will be granted two warrants to acquire up to an additional 10% equity interest, at a premium to market, based on additional targets being introduced into the collaboration. The parties have also agreed to enter into a supply contract, under which uniQure will undertake the manufacturing of all gene therapy products under the collaboration. The Company will also be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead \$100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration, as well as net sales based milestone payments and tiered single to double-digit royalties on product sales.

On April 15, 2015 the Company announced the closing of its follow-on public offering of 3,000,000 ordinary shares at price to the public of \$29.50 per ordinary share. After deducting underwriting discounts but before share issuance expenses, the net proceeds of the follow-on public offering were \$83.2 million ( $\in$ 78.5 million). The securities were offered pursuant to a shelf registration statement on Form F-3 filed with the Securities Exchange Commission (the "SEC") on March 3, 2015 and declared effective on March 13, 2015.

On June 10, 2015, the shareholders of uniQure approved the equity component of the BMS collaboration, and the Company will issue an initial tranche of 1,112,300 ordinary shares to BMS in June for aggregate consideration of \$37.6 million.

The Unaudited Condensed Consolidated Financial Statements were authorized for issue by the supervisory board on June 10, 2015.

#### 2. Summary of Significant Accounting Policies

### 2.1 Basis of Preparation

These Unaudited Condensed Consolidated Financial Statements of the Company were prepared in accordance with International Accounting Standard 34, "Interim Financial Reporting". Certain information and disclosures normally included in Consolidated Financial Statements prepared in accordance with International Financial Reporting Standards ("IFRS") have been condensed or omitted. Accordingly, these Condensed Consolidated Financial Statements should be read in conjunction with the Company's Annual Consolidated Financial Statements for the year ended December 31, 2014 which have been prepared in accordance with IFRS as issued by the International Accounting Standards Board. These Consolidated Financial Statements for the year ended December 31, 2014 were filed with the SEC on April 7, 2015 as part of the Company's Annual Report on Form 20-F.

The preparation of Financial Statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying the Company's accounting policies. The areas involving a higher degree of judgment or complexity, or areas where assumptions and estimates are significant to these Condensed Consolidated Financials are disclosed in Note 4.

The Company's Consolidated Financial Statements are presented in thousands of Euro, which is the Company's presentation currency. Assets and liabilities of Group entities are translated into euro at the period end rates of exchange, and the results of their operations are translated into euro at average

rates of exchange for the period. The resulting translation adjustments are included in accumulated Other Comprehensive Income/ (loss).

#### 2.2 Changes in Accounting Policy and Disclosures

The accounting policies adopted are consistent with those of the previous financial year, except as described below.

In the three months ended March 31, 2015 the Company incurred transaction costs related to the anticipated issuance of equity instruments; the Company deferred these costs on the balance sheet until such a time when the equity transaction would be recognized. Upon closing of the equity transaction the deferred costs will be reclassified as a deduction from equity. If the equity instruments are not subsequently issued, the transaction costs will be recognized as an expense.

#### a) New and amended standards adopted by the Company

There are no standards and amendments to standards that became effective for annual periods beginning on January 1, 2015.

## b) New and amended standards not yet adopted by the Company

| IFRS 9                          | Financial Instruments   |
|---------------------------------|---|
| IFRS 10, 12 and IAS 28          | Amended / Investment Entities; Applying the Consolidation Exception               |
|                                 | Amended / Sale or Distribution of Assets between Investor and its Associate or JV |
| IFRS 11                         | Amended / Accounting for Acquisitions of Interests in Joint Operations            |
| IFRS 14                         | Regulatory Deferral Accounts  |
| IFRS 15                         | Revenue from Contracts With Customers   |
| IAS 1                           | Amended / Disclosure Initiative   |
| IAS 16 and 38                   | Amended / Clarification of Acceptable methods of Depreciation and Amortization    |
| IAS 16 and 41                   | Amended / Agriculture / Bearer Plants   |
| IAS 19                          | Amended / Defined Benefit Plans / Employee Contributions                          |
| IAS 27                          | Amended / Equity Method in Separate Financial Statements                          |
| Improvements to 2012-2014 cycle | Amendments to IFRS5, IFRS 7, IAS19 and IAS 34                                     |

The above standards will not become effective in 2015; the Company has not early adopted any of the above amendments or new standards and has yet to assess the full impact.

#### 3. Financial risk management

#### 3.1 Financial risk factors

The Company's activities expose it to a variety of financial risks: market risk (including currency risk, fair value interest rate risk, cash flow interest rate risk and price risk), credit risk and liquidity risk.

The Condensed Consolidated Financial Statements do not include all financial risk management information and disclosures required in the Annual Consolidated Financial Statements, and should be read in conjunction with the Company's Annual Consolidated Financial Statements for the period ended December 31, 2014.

Since December 31, 2014, other than the employment of the new CFO in January 2015, there have been no material changes in the Company's finance department, which is responsible for financial risk management, nor in the Company's financial risk management policies.

The table below analyzes the Company's financial liabilities in relevant maturity groupings based on the length of time until the contractual maturity date, as at the balance sheet date. The amounts disclosed in the table are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying value balances as the impact of discounting is not significant.

|                                 | LESS THAN<br>1 YEAR | BETWEEN 1<br>AND 2 YEARS | BETWEEN 2<br>AND 5 YEARS | OVER 5<br>YEARS | UNDEFINED |
|---------------------------------|---------------------|--------------------------|--------------------------|-----------------|-----------|
|                                 |                     | (€ in tho                | usands)                  |                 |           |
| At December 31, 2014            |                     |                          |                          |                 |           |
| Borrowings (excl. Finance lease |                     |                          |                          |                 |           |
| liabilities)                    | 1,710               | 7,773                    | 11,480                   | _               | _         |
| Financial lease liabilities     | 168                 | 134                      | —                        | —               | —         |
| Trade and other payables        | 9,449               | —                        | —                        | —               | —         |
| Contingent consideration        | —                   | —                        | —                        | —               | 14,500    |
| Derivatives                     | 852                 | —                        | —                        | —               | —         |
| Total                           | 12,179              | 7,907                    | 11,480                   |                 | 14,500    |
| At period ended March, 2015     |                     |                          |                          |                 |           |
| Borrowings (excl. Finance lease |                     |                          |                          |                 |           |
| liabilities)                    | 3,161               | 7,773                    | 9,608                    | —               | —         |
| Financial lease liabilities     | 171                 | 90                       | —                        | —               | —         |
| Trade and other payables        | 9,174               | —                        | —                        | —               | —         |
| Contingent consideration        | —                   | —                        | —                        | —               | 14,500    |
| Derivatives                     | 2,319               |                          |                          |                 |           |
| Total                           | 14,825              | 7,863                    | 9,608                    |                 | 14,500    |

For financial instruments that are measured on the balance sheet at fair value, IFRS 7 requires disclosure of fair value measurements by level of the following fair value measurement hierarchy:

- Quoted prices (unadjusted) in active markets for identical assets or liabilities (level 1);
- Inputs other than quoted prices included within level 1 that are observable for the asset or liability, either directly (that is, as prices) or indirectly (that is, derived from prices) (level 2); and
- · Inputs for the asset or liability that are not based on observable market data (that is, unobservable inputs) (level 3).

The fair value of financial instruments that are not traded in an active market (for example, over-the-counter derivatives) is determined by using valuation techniques. These valuation techniques maximize the use of observable market data where it is available and rely as little as possible on entity specific estimates. If all significant inputs required to ascertain the fair value of an instrument are observable, the instrument is included in level 2. If one or more of the significant inputs is not based on observable market data, the instrument is included in level 3.

Following the Company's Initial Public Offering in February 2014, the measurement for warrants is now a level 2 valuation, as the Company's shares are traded on NASDAQ and the valuation of warrants is derived from the quoted share price.

The InoCard transaction as described under Note 9 created a level 3 type contingent consideration, as the inputs to calculate this number are not based on observable market data. Details regarding the valuation of the contingent consideration are disclosed in Note 9.

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The carrying amount of a financial asset or financial liability is a reasonable approximation of the fair value and therefore information about the fair values of each class has not been disclosed.

|   | LEVEL 1 | LEVEL 2    | LEVEL 3 | TOTAL |
|---|---------|------------|---------|-------|
|   |         |            |         |       |
| At December 31, 2014                        |         |            |         |       |
| Debt to related party—derivative (warrants) | —       | 645        | —       | 645   |
| Borrowings—derivative (warrants)            | —       | 207        | —       | 207   |
| Contingent consideration                    | —       | —          | 1,454   | 1,454 |
|   |         | 852        | 1,454   | 2,306 |
|   |         |            |         |       |
|   | LEVEL 1 | LEVEL 2    | LEVEL 3 | TOTAL |
|   |         | (€ in thou | sands)  |       |
| At March 31, 2015                           |         |            |         |       |
| Debt to related party—derivative (warrants) | —       | 1,829      | —       | 1,829 |
| Borrowings—derivative (warrants)            | _       | 490        | _       | 490   |
| Contingent consideration                    | —       | —          | 1,251   | 1,251 |
|   |         | 2,319      | 1,251   | 3,570 |
|   |         |            |         |       |
|   |         | LEVEL 3    |         |       |

|   | derivatives at<br>fair value<br>through<br>profit or loss | Contingent<br>consideration<br>(€ in thousands) | Total<br>Level 3 |
|---|---|---|------------------|
| Opening balance January 1, 2014               | 939   | · · · ·   | 939              |
| Transfers to (from) level 3                   | (939)   | _   | (939)            |
| Acquisition of InoCard GmbH (note 9)          | _   | 1,301   | 1,301            |
| (Gains) / Losses recognized in profit or loss | —   | 153   | 153              |
| Closing balance at December 31, 2014          |   | 1,454   | 1,454            |

|   | LEVEL3        | l       |
|---|---------------|---------|
|   | Contingent    | Total   |
|   | consideration | Level 3 |
|   | (€ in thousa  | inds)   |
| Opening balance January 1, 2015               | 1,454         | 1,454   |
| Transfers to (from) level 3                   | —             | —       |
| Acquisition of InoCard GmbH (note 9)          | _             | —       |
| (Gains) / Losses recognized in profit or loss | (203)         | (203)   |
| Closing balance at March 31, 2015             | 1,251         | 1,251   |

At the reporting date the updated valuation of the contingent consideration resulted in a gain of  $\leq 203,000$  that was subsequently taken as a credit to research and development expense through the profit and loss accounts of the Company.

This classification was determined on the basis that the movements in fair value should follow the nature and purpose of the contingent consideration, arising from achieving operational milestones in the further development of the underlying product. The change in valuation of the contingent consideration is caused by a change in the estimate of the timelines of the underlying

#### Group valuation processes

The fair value of the level 2 liabilities as of March 31, 2015 has been determined using a Black-Scholes option pricing model. Key inputs include the risk-free rate, volatility, term, exercise price, and fair value of ordinary shares. The values are included within the tables presented above. Changes in the fair values are analyzed at each reporting date during the quarterly review process. The fair value of ordinary shares is the quoted price as of March 31, 2015.

The fair value of the level 3 liabilities as of March 31, 2015 has been calculated using a Net Present Value calculation; key inputs were the probability of success of achieving the various milestones as well as the time at which they were estimated to have been achieved.

#### 4. Critical Accounting Estimates and Judgments

The preparation of Financial Statements in conformity with IFRS requires the Company to make estimates and assumptions that affect the reported amounts and classifications of assets and liabilities, revenues and expenses in the Condensed Consolidated Interim Financial Statements. The estimates that have a significant risk of causing a material adjustment to the Financial Statements include those utilized for share-based compensation, income taxes, research and development expenditures, borrowings, impairment of goodwill and in-process R&D, fair value of derivatives and other financial instruments. Actual results could differ materially from those estimates and assumptions.

The preparation of Financial Statements in conformity with IFRS also requires the Company to exercise judgment in applying the accounting policies. Critical judgments in the application of the Company's accounting policies relate to research and development expenditures, revenues and the cost of license revenues.

The Condensed Consolidated Financial Statements do not include all disclosures for critical accounting estimates and judgments that are required in the Annual Consolidated Financial Statements, and should be read in conjunction with the Company's Annual Consolidated Financial Statements for the period ended December 31, 2014.

#### **Revenue recognition**

The Company has not generated any revenues from royalties or product sales through March 31, 2015.

In July 2013, the Company received upfront payments in connection with the Glybera commercialization agreement and hemophilia B codevelopment agreements. Revenues from such non-refundable, up-front payments are initially reported as deferred revenues on the Consolidated Balance Sheet and are recognized in revenues as earned over the period of the development, commercialization, collaboration or manufacturing obligation.

The Company also generates revenues from collaborative research and development arrangements. Such agreements may consist of multiple elements and provide for varying consideration terms, such as up-front, milestone and similar payments, which require significant analysis by management in order to determine the appropriate method of revenue recognition.

Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period. Where the arrangement cannot be divided into separate units, the individual deliverables are combined as a single unit of accounting and the total arrangement consideration is recognized over the estimated collaboration period. Such analysis requires considerable estimates and judgments to be made by management, including the relative fair values of the various elements included in such agreements and the estimated length of the respective performance periods.

Management has concluded that the up-front payments constitute a single unit of accounting and, accordingly, the up-front payments will be recognized over the estimated remaining period of the related manufacturing technologies.

## 5. Seasonality of Operations

The Company's financial results have varied substantially, and are expected to continue to vary, from quarter to quarter. The Company therefore believes that period-to-period comparisons should not be relied upon as indicative of future financial results. The Company believes that its ordinary activities are not linked to any particular seasonal factors.

### 6. Segment Information

Operating segments are identified on the basis of whether the allocation of resources and/or the assessment of performance of a particular component of uniQure's activities are regularly reviewed by uniQure's chief operating decision maker as a separate operating segment. By these criteria, the activities of uniQure are considered to be one segment, which comprises the discovery, development and commercialization of innovative gene-therapies, and the segmental analysis is the same as the analysis for uniQure as a whole. The Management Board is the chief operating decision maker, and it reviews the Consolidated Operating Results

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regularly to make decisions about the Company's resources, and to assess overall performance. The acquisition of InoCard GmbH has not changed the Company's assessment of having only one operating segment.

The Company currently derives, and in the near future is expected to derive, the substantial majority of its revenues from a single party, Chiesi, based in Italy. The Company and Chiesi have entered into an exclusive collaboration for the development and commercialization of the Company's Glybera and Hemophilia B programs in Europe and certain additional territories, pursuant to agreements which were entered into in April 2013, and which became effective in June 2013.

## 7. Property, Plant and Equipment

|                  | LEASEHOLD<br>IMPROVEMENTS | CONSTRUCTION<br>IN PROCESS | LAB<br>EQUIPMENT | OFFICE<br>EQUIPMENT | TOTAL |
|------------------|---------------------------|----------------------------|------------------|---------------------|-------|
| (€ in thousands) |                           |                            |                  |                     |       |

| As of January 1, 2015        |         |       |         |         |         |
|------------------------------|---------|-------|---------|---------|---------|
| Cost                         | 15,074  | 884   | 7,200   | 2,544   | 25,702  |
| Accumulated depreciation     | (1,692) | —     | (3,119) | (1,224) | (6,035) |
| Opening net book amount      | 13,382  | 884   | 4,081   | 1,320   | 19,667  |
| Reclassifications            | 30      | (966) | 936     |         |         |
| Additions                    |         | 60    | 1,449   | 9       | 1,518   |
| Disposals                    | —       | —     | (129)   |         | (129)   |
| Depreciation charge          | (415)   | —     | (96)    | (386)   | (897)   |
| Currency translation effects | 1,561   | 81    | 323     | 315     | 2,280   |
| Closing net book amount      | 14,558  | 59    | 6,564   | 1,258   | 22,439  |
| At March 31, 2015            |         |       |         |         |         |
| Cost                         | 16,765  | 59    | 9,773   | 2,654   | 29,251  |
| Accumulated depreciation     | (2,207) | —     | (3,338) | (1,396) | (6,941) |
| Depreciation on disposals    | —       | —     | 129     | —       | 129     |
|                              |         |       |         |         |         |
| Net book amount              | 14,558  | 59    | 6,564   | 1,258   | 22,439  |

Construction in Process ("CIP") at March 31, 2015 relates to the continued build-out of the Company's manufacturing facility in Lexington, Massachusetts.

Depreciation expense of  $\notin$  897,000 for the three months ended March 31, 2015 (three months ended March 31, 2014:  $\notin$  144,000) has been mainly charged to research and development expense.

## 8. Intangible Assets

|                              | LICENSE<br>FEES | CAPITALIZATION<br>OF DEVELOPMENT<br>EXPENSES | IN-PROCESS<br>RESEARCH &<br>DEVELOPMENT | GOODWILL | TOTAL<br>INTANGIBLE<br>ASSETS |
|------------------------------|-----------------|--|---|----------|-------------------------------|
|                              |                 | (€ in  | n thousands)                            |          |                               |
| As of January 1, 2015        |                 |  |   |          |                               |
| Cost                         | 4,892           | 6,811  | 4,665                                   | 1,342    | 17,710                        |
| Accumulated amortization and |                 |  |   |          |                               |
| impairment                   | —               |  | —                                       | —        | —                             |
| Opening net book amount      | 4,892           | 6,811  | 4,665                                   | 1,342    | 17,710                        |
| Additions                    |                 | 1,105  |   |          | 1,105                         |
| Reductions                   | —               | —  | —                                       |          | —                             |
| Amortization charge          | —               | _  | —                                       | —        | —                             |
| Closing net book amount      | 4,892           | 7,916  | 4,665                                   | 1,342    | 18,815                        |
| At March 31, 2015            |                 |  |   |          |                               |
| Cost                         | 4,892           | 7,916  | 4,665                                   | 1,342    | 18,815                        |
| Accumulated amortization and |                 |  |   |          |                               |
| impairment                   | —               | _  | _                                       |          | —                             |
| Net book amount              | 4,892           | 7,916  | 4,665                                   | 1,342    | 18,815                        |

Additions to intangible assets for the three months ended March 31, 2015 include the continued capitalization of Glybera development expenses, in accordance with IAS 38, for a total amount of  $\notin$ 1,105,000 compared with  $\notin$ 1,002,000 for the three months ended March 31, 2014. Capitalization of Glybera costs commenced on March 21, 2013 and had a balance of  $\notin$ 7,916,000 as of March 31, 2015.

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The acquisition of InoCard GmbH (later renamed to uniQure GmbH) in July 2014 as described under note 1, resulted in a total addition of  $\pounds$ 6,007,000, of which  $\pounds$ 1,342,000 is recognized as Goodwill and  $\pounds$ 4,665,000 as in-process research and development.

This goodwill is derived from the potential of adding a new therapeutic area to the current manufacturing platform. Goodwill will be tested annually for impairment following the rules in IAS 36 Impairment of Assets. An asset is impaired when its carrying amount is greater than its recoverable amount. The acquisition does not affect the current segmentation for reporting purposes and the goodwill is therefore assigned to the sole existing segment. The goodwill is expected to be non-deductable for tax purposes.

### 9. Business Combinations

On July 15, 2014 the Company signed and on July 31, 2014 the Company closed an agreement to acquire all shares of InoCard GmbH. InoCard (later renamed uniQure GmbH) was founded in December 2013 as a spin-off of the University of Heidelberg, and is an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy through preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

Under the terms of the agreement, InoCard shareholders have received an upfront payment of approximately &3,000,000 (&1,500,000 in cash and &1,500,000 in uniQure shares), and will receive a further &14,500,000 in success-based milestone payments upon achieving certain clinical and regulatory targets. Upon a successful commercial launch of a developed product, the sellers will further receive a royalty payment of 0.5 % of the net product sales. The amount of the &14,500,000 in milestones is payable, at the Company's sole discretion, in either cash or a variable number of Company shares, based on the then current stock price.

The acquired entity, InoCard, is effectively a single-product business, fully focusing on the further development of gene therapy approaches for cardiac disease. All success based milestones relate to the further development of these programs and therefore these programs are deemed the only material

asset of the entity and as such, the value of InoCard is assumed to fully be represented by the Fair Value of the S100A1 program. As of the acquisition date the Company performed a purchase price allocation under IFRS 3, that resulted in a Fair Value assessment of the acquired IPR&D asset in a value of  $\notin$ 4,665,000.

In determining the fair value of IPR&D, the Company utilized the Income Approach (Discounted Cash Flow method). Inputs to this model were assumptions on pricing and market share developments, together with assumptions on the cumulative probability of success of progressing through the various clinical development stages up to market approval; this method resulted in a series of future cash flow that were discounted at a rate of 30%.

The following table summarizes the consideration paid for InoCard and the amounts of the assets acquired and liabilities assumed, recognized at the acquisition date:

|   | July 31, 2014<br>(€ in thousands) |
|---|-----------------------------------|
| Consideration paid:                                       |                                   |
| Cash paid   | 1,463                             |
| Shares  | 1,500                             |
| Shares issued upon conversion of assumed convertible loan | 17                                |
| Contingent consideration                                  | 1,301                             |
| Total consideration                                       | 4,281                             |

Recognized amounts of identifiable assets acquired and liabilities assumed were as follows:

|                                    | July 31, 2014<br>(€ in thousands) |
|------------------------------------|-----------------------------------|
| Non-current assets                 |                                   |
| Intangible assets (excl. Goodwill) | 4,665                             |
| Current assets                     |                                   |
| Cash and cash equivalents          | 373                               |
| VAT receivable                     | 13                                |
| Non-current liabilities            |                                   |
| Deferred tax liabilities           | (1,379)                           |
| Current liabilities                |                                   |
| Trade payables                     | (7)                               |
| Other payables                     | (726)                             |
| Total identifiable net assets      | 2,939                             |
| Goodwill                           | 1,342                             |
|                                    |                                   |

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The Fair Value of the contingent consideration is estimated as the expected (i.e. probability-weighted) present value of the milestone payments and based on a discount rate of 30 %. The relatively high discount rate is derived from the high uncertainty of progressing from the current pre-clinical development stage trough the various clinical stages before arriving at a commercial stage. The Fair Value of this Contingent Consideration will be remeasured every reporting date with changes recognized in profit & loss for the period. The fair value could change as the probability of the milestone payments changes, or due to the time value of money. The contingent consideration calculated as  $\leq 1,301,000$  is accounted for as a liability. The maximum, undiscounted contingent consideration amounts to  $\leq 14,500,000$  upon achieving clinical milestones with an additional 0.5 % royalty of future net product sales.

The IPR&D is not recognized for tax purposes; therefore a deferred tax liability is recognized for this temporary difference. The deferred tax liability is based on the fair value of the IPR&D multiplied by the German tax rate of 29.58%, resulting in a deferred tax liability of  $\in$ 1,379,000.

## **10. Other Non-Current Assets**

As of December 31, 2014 and March 31, 2015, the amount represents a refundable security deposit for the Lexington, Massachusetts facility, paid in September 2013.

## 11. Trade and Other Receivables

|                                  | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|----------------------------------|----------------------|-------------------|
|                                  | (€ in thous          | sands)            |
| Receivables from related parties | 2,426                | 2,445             |
| Other receivables                | 588                  | 387               |
| Prepaid Expenses                 | 515                  | 1,182             |
| Social security and other taxes  | 439                  | 366               |
| Trade and other receivables      | 3,968                | 4,380             |

The fair value of trade and other receivables approximates their carrying value. As of March 31, 2015 and December 31, 2014, all trade and other receivables were assessed as fully recoverable. The carrying amount of the Company's trade receivables are denominated in Euro and US Dollars.

The receivables from related parties as of March 31, 2015 relate to amounts payable by Chiesi of  $\pounds$ 2,422,000. The remaining element of receivables from related parties relate to certain wage tax liabilities settled by AMT on behalf of senior management in connection with purchases of AMT depositary receipts in 2007; these amounts are repayable to uniQure on sale of the related ordinary shares or on the respective employee ceasing to be employed by the Company of  $\pounds$ 2,000.

Under Prepaid Expenses the Company recorded an amount of €165,000 of deferred transaction costs, related to an anticipated issuance of equity instruments that took subsequently place in April 2015.

The other classes within trade and other receivables do not contain impaired assets. The maximum exposure to credit risk at the reporting date is the carrying value of each class of receivable mentioned above.

The other receivables primarily relate to prepaid rent, insurance and certain annual license fees for software and Intellectual Property.

#### 12. Inventories

|   | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|---|----------------------|-------------------|
|   | (€ in thou           | usands)           |
| Raw materials                           | 152                  | 154               |
| Work in process / Intermediate Products | 48                   | —                 |
| Inventories                             | 200                  | 154               |

Inventories as of March 31, 2015 were  $\leq 154,000$  (December 31, 2014:  $\leq 200,000$ ). The amount includes the raw materials that are capitalized in connection with the manufacturing of Glybera for commercial sale. The reduction in 2015 relates to the expiry of commercially available product.

### 13. Cash and Cash Equivalents

|                          | DECEMBER 31,<br>2014 | MARCH 31,<br>2015 |
|--------------------------|----------------------|-------------------|
|                          | (€ in thou           | sands)            |
| Cash at bank and on hand | 53,219               | 43,197            |

As of March 31, 2015, the Company had  $\notin$  43.2 million of cash on hand, compared to  $\notin$  53.2 million as of December 31, 2014. The change primarily reflects the use of cash to fund operations and investments during the quarter, offset by a foreign exchange gain.

### Supplemental information relating to the cash flow statement

Purchases of fixed assets and changes in trade and other payables exclude a non-cash item of €250,000 principally related to the purchase of fixed assets, which have not yet been paid as of March 31, 2015. Refer to Note 7 above.

Purchases of intangible fixed assets and changes in trade and other payables exclude a non-cash item of €336,000 which has not yet been paid as of March 31, 2015. Refer to Note 8 above.

## 14. Equity

Following the IPO in February 2014 where the Company issued 5,400,000 ordinary shares, as of March 31, 2015, a total of 18,428,866 shares were issued and paid up in full at a nominal value of  $\pounds$ 0.05 per share (December 31, 2014: 18,092,194 shares at  $\pounds$ 0.05 per share).

| Date                    | Description             | Number of<br>Shares | Share Capital<br><u>Amounts</u><br>(€ in thousands) | Share Premium<br><u>Amounts</u><br>(€ in thousands) | Total Equity<br><u>Amounts</u><br>(€ in thousands) |
|-------------------------|-------------------------|---------------------|---|---|--|
| January 1, 2014         | Brought forward         | 12,194,906          | 610   | 142,459   | 143,069  |
| February 5, 2014        | Initial Public Offering | 5,400,000           | 270   | 61,683  | 61,953   |
| July 31, 2014           | Issuance of shares      | 192,128             | 10  | 1,507   | 1,517  |
| September-December 2014 | Exercise of options     | 305,160             | 15  | 462   | 477  |
| January-March 2015      | Exercise of options     | 336,672             | 16  | 131   | 147  |
| March 31, 2015          |                         | 18,428,866          | 921   | 206,242   | 207,163  |

On February 5, 2014 the Company issued 5,400,000 ordinary shares at an initial public offering price of \$17.00 per share. For the issuance of the 5,400,000 ordinary shares, the Company received proceeds, after deducting underwriting discounts but prior to deducting offering expenses payable by the Company, of  $\leq 62,621,000$  (\$85,400,000).

On July 31, 2014 the Company issued 192,128 shares as part of the acquisition price of InoCard GmbH. For further details refer to Note 9.

In the first three months of 2015 the Company issued 336,672 ordinary shares upon exercise of employee share options and options under the 4D plan.

On December 31, 2014 and March 31, 2015 a total of 7,258 shares were held by the stichting participatie AMT as treasury shares. All shares issued by the Company were fully paid. Besides the minimum amount of share capital to be held under Dutch law, there are no distribution restrictions applicable to equity of the Company.

### **Share Premium**

Total additions to share premium during the three months ended March 31, 2015 were €131,000 net of costs. This increase in share premium was due to the issue of shares as described above.

#### **Other Reserves**

The costs of equity-settled share-based payments to employees are recognized in the income statement, together with a corresponding increase in equity during the vesting period, taking into account (deferral of) corporate income taxes.

During the three months ended March 31, 2015 the Company recognized a share-based payment expense of  $\pounds$ 1,809,000 (three months ended March 31, 2014:  $\pounds$ 2,343,000), as described in Note 21. The amount presented in the first three months of 2014 took into account the accelerated vesting of options upon closing of the IPO, as well as the expenses incurred in relation to the granting of options to the management of 4D Molecular Therapeutics. The amount presented for the first three months of 2015 reflects in addition the expenses associated with the 2014 Option Plan that had grant dates of May 27, 2014, October 15, 2014 and January 15, 2015.

During the period the Company recognized €1,371,000 as translation adjustments concerning uniQure Inc., these adjustments are included in the income statement under "Other Comprehensive Income".

For the period presented in these Unaudited Consolidated Financial Statements the Company has legally restricted reserves for the capitalization of Development Costs of  $\notin$ 7,916,000 (2014:  $\notin$ 4,102,000) and for a Currency Translation Adjustment of  $\notin$ 1,371,000 (2014:  $\notin$ 1,000). Only the Currency Translation Adjustment is reflected in the Company's equity.

### **15. Trade and Other Payables**

|                                 | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|---------------------------------|----------------------|-------------------|
|                                 | (€ in thous          | ands)             |
| Trade payables                  | 4,860                | 3,672             |
| Social security and other taxes | 963                  | 945               |
| Other current liabilities       | 3,794                | 4,728             |
|                                 |                      |                   |
| Total trade and other payables  | 9,617                | 9,345             |

#### Other current liabilities

As of March 31, 2015 and December 31, 2014, other current liabilities consisted principally of accruals for services provided by vendors but not yet billed, reimbursements received from research and development partners for expenses which have yet to be incurred and miscellaneous liabilities.

#### 16. Borrowings

|                                  | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|----------------------------------|----------------------|-------------------|
|                                  | (€ in thou           | sands)            |
| Non-current                      |                      |                   |
| Borrowings                       | 16,418               | 17,050            |
| Total non-current                | 16,418               | 17,050            |
| Current                          |                      |                   |
| Debt to related party-derivative | 645                  | 1,829             |
| Borrowings                       |                      | 1,402             |
| Borrowings-derivative            | 207                  | 490               |
| Total current                    | 852                  | 3,721             |
| Total                            | 17,270               | 20,771            |
|                                  |                      |                   |

#### **Borrowings - Derivative**

In 2013 the Company fully converted a convertible loan; the warrants associated with the convertible loan, and which survived the conversion of the loan, are presented in the Consolidated Balance Sheet as at March 31, 2015 within liabilities as a derivative with a fair value of €1,390,000.

#### **Hercules Borrowing**

The presented non-current borrowings relate to the Hercules Technology Growth Corp. venture debt loan facility, entered into on June 14, 2013 for a book value of  $\xi$ 7,062,000 as of June 30, 2014, presented net of expenses for facility charges of 1.25% plus expenses related to legal counsel. The loan commitment was \$10,000,000 with an interest rate of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%, which matures over a period of 39 months from the loan closing date. The interest-only period was initially set at 9 months and was extended to 15 months on completion of the transaction with Chiesi. In addition, the loan is secured by a lien on all of the Company's assets (excluding intellectual property).

During the three months ended March 31, 2015, an amount of \$580,000 (€504,000), compared with \$348,000 (€254,000) for the three months ended March 31, 2014, was recorded as finance expense in relation to the Hercules borrowing.

The warrant included in this loan agreement is not closely related to the host contract and therefore has been split and accounted for separately as a financial derivative measured at fair value though profit or loss. The fair value of this derivative is €490,000 and is included within the Current liabilities: Borrowings—derivative on the Consolidated Balance Sheet as of March 31, 2015.

On June 26, 2014 the Company entered into an amended and restated loan agreement (which amends and replaces the original loan agreement) of 20,000,000 (then 14,600,000), presented net of expenses for facility charges of 1.00% plus expenses related to legal counsel. The additional amount of 10,000,000 (7,344,000) was received net of expenses of 218,000 (160,000). The net cash inflow was 9,782,000 (7,184,000). The total loan

commitment is \$20,000,000 with an interest rate of 10.25%. Also included are two back-end fees of \$345,000 and \$250,000, due October 2016 and June 2018 respectively. The interest-only period is 18 months. The Company is required to repay the loan in monthly principal installments from January 2016 through June 2018. As the terms of the amended loan agreement changed significantly compared to the original loan agreement (maturity date, interest rate, payback schedule), the Company fully amortized the unamortized transaction costs at issue, resulting in an extra amortization charge through profit and loss in 2014 of \$193,000 ( $\in$ 141,000).

The total value for the amended loan as of March 31, 2015 is 20.0 million (18.4 million) and is recorded net of expenses under non-current borrowings. The warrants included in the original loan agreement remain in place and are unaffected. The fair value of the borrowings equals their carrying amount, as the impact of discounting is insignificant as the loan is already amortised at a market conforming interest rate.

The foreign exchange expense on the borrowings was  $\leq 2.0$  million for the period ending March 31, 2015. In the period ended March 31, 2015 the current element of this loan facility amounted to \$1.5 million ( $\leq 1.4$  million).

The Amended Loan and Security Agreement contains covenants that restrict the Company's ability to, among other things, incur future indebtedness and obtain additional financing, to make investments in securities or in other companies, to transfer assets, to effect certain corporate changes, to make loans to employees, officers and directors, and to make dividend payments and other

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distributions. Further, the Company has periodic reporting requirements and is required to keep a minimum cash balance deposited in bank accounts in the United States, equivalent to the lesser of the outstanding balance of principal due and 50% of the Company's worldwide cash reserves. This restriction on the cash reserves only relates to the location of the cash reserves, but all cash reserves are at the free disposal of the Company. The Amended Loan and Security Agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by the Company immediately due and payable. As of March 31, 2015, the Company was in compliance with these covenants in all material respects.

## **Finance Lease Liability**

The finance lease liability relates to the Company's facility at Meibergdreef in Amsterdam, the Netherlands.

The Condensed Consolidated Financial Statements do not include all disclosures for borrowings that are required in the Annual Consolidated Financial Statements, and should be read in conjunction with the Company's Annual Consolidated Financial Statements for the period ended December 31, 2014.

#### **17. Revenues and Deferred Revenues**

|                                   | MARCH, 31<br>2014    | MARCH, 31<br>2015 |
|-----------------------------------|----------------------|-------------------|
|                                   | (€ in thou           | sands)            |
| License revenues                  | 220                  | 221               |
| Collaboration revenues            | 950                  | 886               |
| Total                             | 1,170                | 1,107             |
|                                   | DECEMBER, 31<br>2014 | MARCH, 31<br>2015 |
|                                   | (€ in thou           | sands)            |
| Deferred revenues current portion | 1,328                | 1,539             |
|                                   |                      |                   |
| Deferred revenues                 | 15,387               | 15,237            |

During the three months ended March 31, 2015, an amount of  $\pounds$ 221,000 (three months ended March 31, 2014:  $\pounds$ 220,000) was recognized as license revenues. This amount relates to the recognition of the up-front payments received from Chiesi. During the three months ended March 31, 2015, an amount of  $\pounds$ 886,000 (three months ended March 31, 2014:  $\pounds$ 950,000) was recognized as collaboration revenues. This amount relates to reimbursements of expenses under the Company's Co-Development Agreement with Chiesi in respect of its Hemophilia B program.

Upon signing of the Commercialization Agreement and the Co-Development and Commercialization Agreement with Chiesi on April 29, 2013, the Company received  $\pounds$ 17,000,000 as a non-refundable upfront payment. Based on an assessment performed by the Company, the  $\pounds$ 17,000,000 will be amortized on a straight-line basis, and presented as license revenues, over a period from July 2013 through September 2032: the date of expiration of the last intellectual property protection related to the Company's manufacturing process. The Company determined that the  $\pounds$ 17,000,000 of up-front payments received from Chiesi constituted a single unit of accounting.

Collaboration revenues from contracts, typically from delivering research and development services are recognized on the basis of labor hours delivered at the agreements' full time employee rate.

Cost reimbursements to which the Company is entitled under agreements are also recognized as collaboration revenues in the income statement in the same quarter of the recorded cost they are intended to compensate.

## 18. Research and Development Expenses

For the three months ended March 31, 2015 the research and development expenses amounted to  $\leq 10,106,000$  (three months ended March 31, 2014:  $\leq 6,218,000$ ). These increases are mainly due to the additional development and clinical activities associated with the Phase I/II study of AMT-060, operations

in the U.S. facility, which came online in the second half of 2014, and the planned commercial launch of Glybera and the continued progression of uniQure's other programs through later stage research and clinical development.

#### **19. General and Administrative Expenses**

For the three months ended March 31, 2015 general and administrative expenses amounted to  $\pounds$ 4,159,000 (three months ended March 31, 2014:  $\pounds$ 2,268,000). These increases are primarily due to increases in expenses related to consultants (commercial and administrative) and professional fees (legal), and an allocation of the increased overall accommodation costs related to the U.S. facility.

#### 20. Other gains & losses

For the three months ended March 31, 2015, in other Gains & Losses a gain of  $\notin$ 4,245,000 is included which represents the foreign exchange result on our USD denominated bank accounts. For the three months ended March 31, 2014 we recorded a loss of  $\notin$ 519,000.

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#### 21. Other Comprehensive Income

For the three months ended March 31, 2015 Other Comprehensive Income amounted to  $\pounds$ 1,371,000 (three months ended March 31, 2014:  $\pounds$ 2,000). The amounts shown represent the foreign currency translation arising from the U.S. subsidiary, which was established in May 2013.

#### 22. Finance Expense

For the three months ended March 31, 2015, Finance Expense amounted to &3,950,000 (three months ended March 31, 2014: &259,000). The &3,950,000 consists of amounts for Interest Expense of &509,000, foreign exchange loss of &1,974,000 on the Hercules loan facility and an amount of &1,467,000 related to the loss on the fair value of the warrants / derivatives.

The  $\pounds$ 259,000 consists of amounts for Interest Expense of  $\pounds$ 262,000, foreign exchange loss of  $\pounds$ 7,000 on the Hercules loan facility and an amount of ( $\pounds$ 10,000) related to a gain on the fair value of the warrants / derivatives.

#### 23. Share-Based Payments

The Condensed Consolidated Financial Statements do not include all disclosures for share-based payments that are required in the Annual Consolidated Financial Statements, and should be read in conjunction with the Company's Annual Consolidated Financial Statements for the period ended December 31, 2014.

Total options outstanding as at March 31, 2015 were 2,915,668.

During the three months ended March 31, 2015 the Company recognized share-based payment expense of €1,809,000 (three months ended March 31, 2014: €2,342,000). The 2015 amount includes an amount of €273,000 related to Restricted Stock Units that were granted in 2014.

### 24. Loss Per Share

#### Basic

Basic loss per share is calculated by dividing the loss attributable to equity holders of the Company by the weighted average number of issued and outstanding ordinary shares during the year.

|  | March 31,<br>2014 | March 31,<br>2015 |
|--|-------------------|-------------------|
|  | (€ in thous       |                   |
| Loss attributable to equity holders of the Company ( $\in$ in thousands) | (7,829)           | (12,638)          |
| Weighted average number of ordinary shares outstanding                   | 15,134,906        | 18,332,137        |
| Loss per Share (€)   | (0.52)            | (0.69)            |

#### Diluted

Diluted loss per share is calculated by adjusting the weighted average number of ordinary shares outstanding, assuming conversion of all dilutive potential ordinary shares. Due to the fact that the Company is loss making, all potential ordinary shares had an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share.

|                                 | March 31,<br>2014 | March, 31<br>2015 |
|---------------------------------|-------------------|-------------------|
| Warrants                        | 170,802           | 170,802           |
| Share options under 2012 Plan   | 1,691,844         | 1,495,982         |
| Share options under the 4D plan | 609,744           | 152,436           |
| Share options under 2014 Plan   |                   | 1,267,250         |
| RSU's                           |                   | 179,068           |
| Total                           | 2,472,390         | 3,265,538         |

#### 25. Related-Party Transactions

In the three month periods ended March 31, 2015 and 2014, the Management Board received regular salaries and contributions to post-employment schemes. Additionally, selected members of the Supervisory Board received compensation for their services in the form of cash compensation.

Following the IPO the Company recognizes as related parties only those shareholders that are holding more than 5% of the Company's shares.

Funds affiliated with Forbion Capital partners have a material interest in the Company. In addition, Professor Sander van Deventer and Mr. Sander Slootweg, who were appointed as members of the Supervisory Board of uniQure on April 5, 2012, are each partners of Forbion. Based on the information above, Forbion is a related party of uniQure.

Funds affiliated with Gilde Healthcare have a material interest in the Company and Gilde Healthcare is considered a related party of uniQure.

Chiesi became a related party following the commercial and investment agreements concluded with the Company on June 30, 2013, and Coller Capital became a related party following the conversion of the convertible loan in July 2013.

#### Transactions

In the period ending March 31, 2015 the Company received various payments from Chiesi for issued invoices totaling  $\leq 1,177,000$ . As of March 31, 2015 the Company had a receivable outstanding with Chiesi for  $\leq 2,306,000$ .

#### **Key Management Compensation**

The below table shows the compensation paid to the Supervisory Board, the Managing Directors and senior Management:

| FOR THE                       | _                  | SHORT TERM<br>EMPLOYEE<br>BENEFITS | SHARE BASED<br>PAYMENTS (1) | POST-<br>EMPLOYMENT<br>BENEFITS | ADVISORS<br>FEE | TERMINATION<br>BENEFITS | TOTAL |
|-------------------------------|--------------------|------------------------------------|-----------------------------|---------------------------------|-----------------|-------------------------|-------|
|                               |                    |                                    |                             | (€ in thousands)                | )               |                         |       |
| Year ended December 31, 2014  | Supervisory Board  | —                                  | 162                         | —                               | 178             | _                       | 340   |
|                               | Managing directors | 646                                | 660                         | 25                              | _               | _                       | 1,331 |
|                               | Senior Management  | 1,791                              | 1,437                       | 208                             | _               | _                       | 3,436 |
|                               | Ŭ                  | 2,437                              | 2,259                       | 233                             | 178             |                         | 5,107 |
| 3 months ended March 31, 2014 | Supervisory Board  | _                                  | 76                          | _                               | 42              |                         | 118   |
|                               | Managing directors | 163                                | 106                         | 11                              |                 |                         | 280   |
|                               | Senior Management  | 348                                | 539                         | 40                              | —               | _                       | 927   |
|                               | Ŭ                  | 511                                | 721                         | 51                              | 42              |                         | 1,325 |
| 3 months ended March 31, 2015 | Supervisory Board  | _                                  | 291                         | _                               |                 |                         | 291   |
|                               | Managing directors | 307                                | 454                         | 15                              | _               | _                       | 776   |
|                               | Senior Management  | 600                                | 226                         | 51                              | —               | _                       | 877   |
|                               |                    | 907                                | 971                         | 66                              |                 |                         | 1,944 |

(1) In the three months ended March 31, 2014, of the total amount, €335,000 related to the accelerated vesting of options upon the closing of the IPO.

The Condensed Consolidated Financial Statements do not include all disclosures for related-party transactions that are required in the Annual Consolidated Financial Statements, and should be read in conjunction with the Company's Annual Consolidated Financial Statements for the period ended December 31, 2014.

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#### 26. Commitments / Contingent Liabilities

uniQure leases various office space and laboratory space under operating lease agreements. The Company leases its headquarters under an agreement between uniQure and AMC, represented by BDDA and Amsterdam Vector Productions B.V. ("AVP"), both subsidiaries of AMC (Second Rental Agreement) in respect of facilities located at Meibergdreef 61 Amsterdam, from October 1, 2005 until September 30 2016, and an agreement for the lease of facilities at Meibergdreef 57, Amsterdam, from July 1, 2006 until September 30, 2016. The aggregate annual lease payments amount to €542,000.

The lease expenditure charged to the income statement for the three months ended March 31, 2015 was  $\leq 133,000$  (for the three months ended March 31, 2014:  $\leq 132,000$ ).

The future aggregate minimum lease payments under non-cancellable operating leases as of March 31, 2015 and December 31, 2014 are as follows:

|   | DECEMBER 31,<br>2014 | MARCH, 31<br>2015 |
|---|----------------------|-------------------|
|   | (€ in thous          | ands)             |
| No later than 1 year                        | 1,918                | 2,097             |
| Later than 1 year and no later than 5 years | 6,394                | 7,032             |
| Later than 5 years                          | 7,285                | 7,717             |
|   |                      |                   |
| Total                                       | 15,597               | 16,846            |

On July 24, 2013 uniQure entered into an agreement for the lease of facilities at 113 Hartwell Avenue, Lexington, Massachusetts, United States that became effective from November 5, 2013 onwards until November 5, 2023. uniQure has an option to extend the lease for up to an additional 10 years. The

aggregate lease payments for the period to November 5, 2023 amount to 18,937,000 (17,278,000), including an initial rent-free period of seven months from the commencement of the lease which was effective at November 5, 2013.

The lease payments under an operating lease will be recognized as an expense on a straight line basis over the full duration of the lease, taking into account the lease incentives for a total of \$7,259,000 ( $\in$ 6,690,000) as received from the landlord. This results in a monthly expense of \$92,680 ( $\in$ 85,411). During 2015 the Company expensed a total amount of \$278,000 ( $\in$ 245,000). As of March 31, 2015 the Company recorded a total deferred rent of \$7,319,000 ( $\in$ 6,745,000), with a current element of \$590,000 ( $\in$ 544,000). Further details regarding the accounting for this lease are set out in the Audited Consolidated Financial Statements for the year ending December 31, 2014.

### **Research and Development Commitments**

uniQure has entered into research and development commitments in relation to uniQure's product pipeline. The future aggregate minimum payments under these research and development commitments are as follows:

|   | DECEMBEF<br>2014 | R 31,        | MARCH, 31<br>2015 |
|---|------------------|--------------|-------------------|
|   |                  | (€ in thousa | ands)             |
| No later than 1 year                        |                  | 306          | 337               |
| Later than 1 year and no later than 5 years |                  | —            | _                 |
| Later than 5 years                          |                  | —            |                   |
|   |                  |              |                   |
| Total                                       |                  | 306          | 337               |
|   |                  |              |                   |
|   | 23               |              |                   |

### **Grant Commitments**

From October 1, 2000 until May 31, 2005, AMT received a technical development loan from the Dutch government in relation to development of Glybera. This grant includes a repayment clause in the event the Company generates revenues from the related project. AMT received total grants of €3,605,000 relating to eligible project costs in the grant period. The grant amount received bears interest of 5.7% per annum and must be repaid in the period January 1, 2008 through December 31, 2017 as a percentage of revenues which are derived from product sales of Glybera. If future royalty payments are not sufficient to repay the grant on or prior to December 31, 2017, or if there are no revenues generated, the remaining balance will be forgiven. Repayment obligations continue to apply if the product is not commercialized or transferred to others. The total amount of the contingent commitment as at March 31, 2015 was €5,904,000 (2013: €5,586,000), comprising the original total amount of the grant together with accrued interest.

Historically, the Company also received a "Technisch ontwikkelingsproject" (TOP) (or technical development project) grant from the Dutch government amounting to €130,000 on a project that was terminated. If the Company realizes income from the sale of assets developed under that grant, repayment clauses will apply. The Company has not recorded any liability to repay amounts in respect of this grant within these Financial Statements.

#### Other contingent liabilities

On December 11, 2013, the Company received a formal request for arbitration from Extera Partners, a consulting firm based in Cambridge, Massachusetts, alleging a fee to be due in respect of consulting services provided to the Company in connection with a partnering transaction. The request for arbitration was received by the International Court of Arbitration at the International Chamber of Commerce on December 12, 2013. The amount claimed is \$100,000 plus 2.5% of all proceeds the Company is due to receive from Chiesi (actual during 2013 and on the NPV of future proceeds), pursuant to its collaboration agreements entered into in the second quarter of 2013. The Company's engagement letter with Extera Partners contains a cap limiting the maximum liability to €5,000,000.

On May 12, 2014, the ICC appointed and confirmed a sole arbitrator. On October 1, 2014, Extera Partners LLC filed its Statement of Case which includes an estimated claim based on the formula mentioned above and on Extera's estimate of potential future revenues. On January 20, 2015 the Company filed its Statement of Defence, followed by a further Statement of Reply dated May 8, 2015 addressing the assumptions in the Extera Statements. A final merits hearing has been scheduled for July 2015. The Company has denied the claim and intends to vigorously defend against it.

#### 27. Events After the Balance Sheet Date

On April 6, 2015, the Company entered into agreements with BMS, which provide BMS exclusive access to uniQure's gene therapy technology platform for multiple targets in cardiovascular and other target-specific disease areas. The collaboration includes the Company's proprietary congestive heart failure gene therapy program, which has demonstrated in advanced preclinical models that it can restore the ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and increase survival rates after myocardial infarction. In addition, the Company will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of cardiovascular and other target-specific disease areas. uniQure will be responsible for discovery, preclinical development, and CMC, and will provide BMS its vector technologies and access to its industrial, proprietary insect-cell based manufacturing platform. uniQure will be responsible for CMC portions of regulatory filings, and will co-operate with BMS in the preparation of all regulatory materials and interactions with regulatory authorities. BMS will be responsible for clinical development and all commercial activities across all programs.

The financial terms consist of guaranteed, near-term payments to uniQure of \$103 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the transaction (other than the initial equity investment) occurred on May 21, 2015. An additional \$15 million payment is to be received following the selection of three additional collaboration targets, in addition to the S100A1 program, within three months of the closing. In addition, an initial equity investment in uniQure will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or about \$38 million in total. This investment is expected to be completed in the second quarter of 2015. BMS is also obligated to make an additional equity investment in uniQure for a number of shares that will equal 5.0% of the total number of shares outstanding following such issuance by December 31, 2015 and will be granted two warrants to acquire up to an additional 10% equity interest, at a premium to market, based on additional targets being introduced into the collaboration. The parties have also agreed to enter into a supply contract, under which uniQure will undertake the manufacturing of all gene therapy products under the collaboration. The Company will also be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead S100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration, as well as net sales based milestone payments and tiered single to double-digit royalties on product sales.

On April 15, 2015 the Company announced the closing of its follow-on public offering of 3,000,000 ordinary shares at price to the public of \$29.50 per ordinary share. After deducting underwriting discounts but before share issuance expenses, the net proceeds of the follow-on public offering were 83.2 million (₹78.5 million). The securities were offered pursuant to a shelf registration statement on Form F-3 filed with the Securities Exchange Commission (the "SEC") on March 3, 2015 and declared effective on March 13, 2015.

On June 10, 2015, the shareholders of uniQure approved the equity component of the BMS collaboration, and the Company will issue an initial tranche of 1,112,300 ordinary shares to BMS in June for aggregate consideration of \$37.6 million.

On April 8, 2015, the Company received a copy of a preliminary assessment report on Glybera prepared by the rapporteur designated by the Committee for Advanced Therapies (CAT), which is the committee that advises the Committee for Human Medicinal Products (CHMP) on gene therapies. The preliminary report was a response to the Company's submission to the European Medicines Agency (EMA) on September 5, 2014 of a Type II variation, which proposed an amendment to the Glybera Summary of Product Characteristics (SPC) to reflect certain information from the six year follow up data included in the Company's final clinical study report. The preliminary assessment report, which represented solely the view of the rapporteur, stated that Glybera lacked efficacy and therefore the benefit-risk balance was negative. The rapporteur's preliminary report was provided to the CAT for further discussion in advance of the CAT's monthly meeting on April 16-17.

On April 24, the Company received a copy of the final assessment report prepared by the CAT and endorsed by the CHMP, which stated the following:

The CAT did not agree with the negative view of the rapporteur and concluded by majority that the efficacy of Glybera needed to be considered in its totality as defined in the initial approval taking into account the criteria considered at time of initial approval.

In accordance with the Company's Type II variation request, the CAT will continue to evaluate the six year follow up data and has requested supplemental information, which the Company submitted on May 22.

On April 28, the Company informed the Federal Joint Committee (G-BA), which is responsible for the commercialization of Glybera in Germany, of its receipt of the final assessment report from the CAT. Previously, the G-BA had put its ongoing benefit assessment of Glybera on hold to await the final assessment of the CAT and the CHMP regarding benefit/risk. Based on the recommendations stated in the final assessment report, the Company had requested the G-BA to resume its benefit assessment of Glybera. On May 21, the G-BA published their final assessment of the additional benefit of Glybera, classifying it as "non-quantifiable". This is in line with uniQure and Chiesi's expectations, as this rating is reserved for drugs that are deemed to have a benefit, but for which insufficient data currently exists (such as is the case with many orphan drugs). This decision now allows Chiesi to proceed to the next phase, typically lasting up to six months, which involves the pricing negotiations with the authorities. The G-BA will re-evaluate the benefit of Glybera on June 1, 2016 if there is any significant new data available. In the meantime, Glybera can be prescribed by physicians in Germany.

The Company continues to believe that the clinical data from its Glybera development program, including the six-year follow-up data, support the long-term value and efficacy. However, the Company can provide no assurance regarding the final conclusions of the EMA and G-BA. Any adverse outcomes could require the Company to expend significant additional resources to support its conclusions or could have a material negative impact on the revenue expectations for Glybera.

On June 10, the Company's shareholders approved certain amendments to the 2014 Share Incentive Plan, including an increase in the authorized number of ordinary shares available for future grant by 1,070,000.

No other events occurred after the balance sheet date that would have a material impact on the result or financial position of uniQure.

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## UNIQURE N.V.

### MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following is a discussion of our financial condition and results of operations for the three months ended March 31, 2015. You should read the following discussion and analysis together with the Unaudited Condensed Consolidated Financial Statements and related notes previously filed by the Company. For additional information relating to our management's discussion and analysis of financial condition and results of operations, please see our annual report on Form 20-F for the year ended December 31, 2014 filed with the U.S. Securities and Exchange Commission on April 7, 2015.

### Overview

uniQure is a leader in the field of gene therapy and has a technology platform that we use as the basis for our proprietary and collaborative product lines across multiple therapeutic areas. Our core gene therapies include AMT-060 for the treatment of hemophilia B, which we expect to enter into a Phase I/II clinical trial in the first half of 2015; S100A1 for the treatment of congestive heart failure, which is at the preclinical/proof of concept phase; and Glybera, the first and currently the only gene therapy product to receive regulatory approval in the European Union.

Our aim is to make gene therapy a mainstay of modern medicine by:

• using our strong technology platform to develop our own programs in three therapeutic areas, liver-based diseases, cardio/metabolic diseases, and central nervous system diseases, in which we have a competitive advantage, with the potential to significantly de-risk development programs, and reduce

development cost and time to market;

sponsoring and acquiring additional early-stage programs in these areas from other biopharmaceutical companies and academic investigators;

• enhancing and accelerating these programs through our modularized research and development platform and our experience of the EU and FDA regulatory environments for gene therapies;

• applying our industrialized manufacturing process to produce the highest-quality material for our own and our collaborators' programs, and

• collaborating with pharmaceutical companies with the necessary expertise to enhance our late-stage therapy development and maximize the value of our therapies at the commercialization stage.

We believe that our technology platform and strategic collaborations place us at the forefront of gene therapy within our chosen therapeutic areas. Our transgene delivery system is based on common, adeno-associated viruses, or AAV, which we believe are safe and effective delivery methods for efficient expression of transgenes. We have the exclusive or non-exclusive rights to natural AAV serotypes for lipoprotein lipase deficiency, or LPLD, liver and CNS applications and the capability to identify and develop synthetic AAV vectors that are designed to optimize the expression of a particular transgene in specific tissue types. We produce our AAV-based vectors in our own facilities with a proprietary, commercial-scale, consistent, and robust manufacturing process using insect cells and baculoviruses, a common family of viruses found in invertebrates. We believe our Lexington, Massachusetts-based facility, which is currently being qualified, is one of the world's largest, most versatile, gene therapy manufacturing facilities. We believe this robust technology platform, combined with our know-how derived from achieving the first regulatory approval of a gene therapy in the European Union, provides us a significant advantage in bringing our gene therapy products to the market ahead of our competitors.

We seek to develop gene therapies targeting a range of liver-based, cardio/metabolic and CNS indications, from ultra-orphan diseases, such as LPLD (for which Glybera is designated), to orphan diseases such as hemophilia B, to common diseases that affect far larger populations, such as congestive heart failure. The core of uniQure is a versatile and universal technology backbone, applicable to multiple therapeutic areas with the potential to significantly derisk development programs, and reduce development cost and time to market. As part of our strategy, we are accessing important medical expertise for our therapeutic focuses through strong ties with academic thought leaders and clinical institutions. For cardio/metabolic diseases we are building a center of expertise in our German subsidiary, uniQure GmbH, in close cooperation with leading academic clinicians and surgeons at the university hospital and heart center in Heidelberg, Germany. Our CNS activities are based on strong collaborations with the University of California at San Francisco, the National Institutes of Health, and the Institut Pasteur, Paris, France. Our hemophilia B product originates from St. Jude Children's research Hospital in Memphis, Tennessee. We also seek to collaborate with or acquire emerging companies within our chosen therapeutic areas that are conducting or sponsoring early-stage clinical trials. Our collaborations allow us to cost-effectively obtain access to pre-clinical and early-stage programs without expending significant resources of our own. We generally have the rights to the data generated in these collaborator-sponsored programs, but do not

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control their design or timing. Our collaboration programs include gene therapy candidates for Parkinson's disease, Sanfilippo B syndrome, Acute Intermittent Porphyria, and amyotrophic lateral sclerosis.

Our business was founded in 1998 by scientists who were investigating LPLD at the Academic Medical Center of the University of Amsterdam, or the AMC. In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. On February 5, 2014, we successfully completed our initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91.8 million ( $\in 67.3$  million) and net proceeds of \$85.4 million ( $\in 62.6$  million) after commissions but before expenses. From our first institutional venture capital financing in 2006 until our initial public offering, we funded our operations primarily through private and public placements of equity securities, and other convertible debt securities, in the aggregate amount of  $\notin 204.5$  million (\$266.7million). During this period, we also received total other income, consisting principally of government grants and subsidies, of  $\notin 6.9$  million, and total nonrefundable collaboration funding of  $\notin 17.0$  million. Our predecessor entity, Amsterdam Molecular Therapeutics (AMT) N.V., or AMT, completed an initial public offering of its ordinary shares on Euronext Amsterdam in 2007 and subsequently delisted from that exchange in 2012. We acquired the business of AMT in the first half of 2012.

As of March 31, 2015, we had cash and cash equivalents of €43.2 million. To date, we have not generated any revenues from royalties or product sales. We do not expect to generate royalty or revenues from product sales prior to the commercial launch of Glybera by Chiesi.

We had a net loss of  $\leq 12.6$  million in the first three months of 2015 and  $\leq 7.8$  million for the same period of 2014. As of March 31, 2015, we had an accumulated deficit of  $\leq 193.7$  million. We anticipate that our expenses will increase substantially in the future as we:

- · conduct a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- expand our research capabilities and corporate infrastructure to support our collaboration with BMS to develop gene therapies in cardiovascular and other target-specific areas;
- · complete our EMA-mandated post-approval clinical trials of Glybera and maintain an LPLD patient registry;
- conduct a clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA;
- seek marketing approval for Glybera in the United States and other countries;
- advance the preclinical and clinical development of our other product candidates, most of which are at relatively early stages of development, and seek to discover and develop additional product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- exercise our options to acquire rights and pursue development of certain product candidates, the development of which is currently being conducted and funded by third parties;
- acquire or in-license rights to new therapeutic targets or product candidates;
- enter into collaboration agreements with third parties to collaborate on the research and development of potential product candidates;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- fund the ongoing operations of our new manufacturing facility in Lexington, Massachusetts;
- fund expenses in connection with our collaboration with 4D Molecular Therapeutics;

- maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties;
- hire additional personnel, particularly in our manufacturing, research, clinical development, medical affairs, commercial and quality control groups; and
- · add operational, financial and management information systems and related finance and compliance personnel.

#### **Collaboration and License Agreements**

## **Chiesi Agreements**

In April 2013, we entered into two collaboration agreements with Chiesi. In July 2013, we received an aggregate of  $\leq 17.0$  million in upfront payments from Chiesi under these agreements, as well as a  $\leq 14.0$  million investment in our ordinary shares.

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#### Glybera agreement

Under the Glybera agreement, we granted Chiesi the exclusive right to commercialize Glybera for LPLD in the European Union and other specified countries, excluding the United States. In July 2013, we received a  $\leq 2.0$  million upfront payment in recognition of our past expenditures incurred in developing the product. In addition, we are eligible to earn up to  $\leq 42.0$  million in commercial milestone payments based on annual sales of Glybera.

We will receive payments for the quantities of Glybera we manufacture and supply to Chiesi, payable in part upon order and in part upon delivery of such product quantities. We will bear the cost of goods sold for the Glybera we deliver, including the royalties and related payments to third parties we must make under the license agreements covering various aspects of the technology underlying the composition and manufacture of Glybera. We estimate that the amount we will retain, net of cost of goods sold, including such third party royalties and related amounts, will be between 20% and 30% of the revenues from sales of Glybera by Chiesi, varying by country of sale. We believe that the amount that we will retain from net sales of Glybera in the European Union will initially be at the lower end of this range and will increase toward the higher end of that range beginning in 2015, upon the expiration of an in-licensed patent on which we pay royalties. In addition, we are required to pay 20% of the gross amount we receive from Chiesi in respect of Glybera product sales to the Dutch government, in repayment of a technical development loan in the outstanding amount of  $\xi$ 5.9 million as of March 31, 2015, until the earlier of repayment in full of such amount and 2019.

#### Hemophilia B agreement

Under the Hemophilia B agreement, we granted to Chiesi an exclusive license, for the European Union and specified countries other than the United States, to co-develop and exclusively commercialize AMT-060, a gene therapy product for the treatment of hemophilia B. We received a €15.0 million upfront payment under this agreement. Of this amount, €5.0 million related to the future development of our hemophilia B product candidate and €10.0 million related to the use of our manufacturing capacity for our hemophilia B product candidate. In addition, we will share equally with Chiesi specified development expenses attributable to the hemophilia B program according to a defined development plan and budget, including expenses associated with preclinical and clinical studies as well as development and regulatory milestone payments associated with existing in-license agreements. We will receive payments from Chiesi for commercial quantities of our hemophilia B product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by country of sale. We and Chiesi have agreed to negotiate a separate supply and distribution agreement in respect of the potential commercialization of our hemophilia B product candidate prior to dosing the first patient in any pivotal study. We are not entitled to any milestone payments under this co-development agreement.

#### Strategic Collaboration: 4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics, or 4D, for the discovery and optimization of next-generation AAV vectors. Under this agreement, we have an exclusive license to 4D's existing and certain future know-how and other intellectual property for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, has established a laboratory, which we are funding at a cost of approximately \$3.0 million in aggregate through 2016, to identify next generation AAV vectors. We are also required to make payments for pre-clinical, clinical and regulatory milestones under the collaboration as well as to pay single-digit royalties. In addition, we have granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense through 2016. To the extent that the collaboration is successful, we may also incur additional third party costs in developing any product candidates and also in preparing, filing and prosecuting additional patent applications.

#### Strategic Collaboration: Treeway

On January 14, 2015 we announced a License and Collaboration Agreement with Treeway to Develop a Gene Therapy for Amyotrophic Lateral Sclerosis (ALS). Treeway is a biotechnology company and was founded by entrepreneurs Bernard Muller and Robbert Jan Stuit, both diagnosed with ALS. Treeway's strategy is founded on a cohesive combination of approaches that together should provide the highest likelihood of bringing successful treatments for ALS to the patient in the short term. Under the terms of the agreement there will be no upfront or milestone payments. Treeway is responsible for the development of the therapy and we are entitled to receive payments for manufacturing as well as commercial rights in North and South America and Japan.

## Strategic Collaboration: Synpromics Ltd

On January 31, 2015 we entered into a collaboration and license agreement with Synpromics Ltd. for the discovery and selection of promoters with improved activity. Under this agreement, uniQure has the exclusive rights to five selected promoter sequences for driving gene expression in liver cells using AAV mediated gene therapy. Synpromics has generated a patent protected technology to create a rationally designed library of DNA fragments which can be

used to assemble synthetic promoters with improved activity. Under the agreement Synpromics and uniQure collaborate in the selection of the promoters using Synpromics' protected technology to create rationally designed libraries of DNA fragments, which can be used to assemble synthetic promoters with superior characteristics. We are required to make payments for pre-clinical, clinical and regulatory milestones under this collaboration as well as low single digit royalties.

### Strategic Collaboration: Bristol Myers Squibb

On April 6, 2015, we entered into agreements with BMS, which provide BMS exclusive access to our gene therapy technology platform for multiple targets in cardiovascular and other target-specific disease areas. The collaboration includes our proprietary congestive heart failure gene therapy program, which has demonstrated in advanced preclinical models that it can restore the ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and increase survival rates after myocardial infarction. In addition, we will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of cardiovascular and other target-specific disease areas. uniQure will be responsible for discovery, preclinical development, and CMC, and will provide BMS its vector technologies and access to its industrial, proprietary insect-cell based manufacturing platform. uniQure will be responsible for CMC portions of regulatory filings, and will co-operate with BMS in the preparation of all regulatory materials and interactions with regulatory authorities. BMS will be responsible for clinical development and all commercial activities across all programs.

#### **Other License Agreements**

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we are exploiting in Glybera and our development programs. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a portion of amounts we receive from our sub-licensees and payments upon the achievement of specified development, regulatory or commercial milestones. Our potential aggregate financial obligations under these agreements are material. Some of the agreements may also specify the extent of the efforts we must use to develop and commercialize licensed products.

#### InoCard Acquisition

In July 2014 we acquired InoCard GmbH (later renamed uniQure GmbH), an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg. InoCard has developed a novel gene therapy through preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

Under the terms of the agreement, InoCard shareholders have received an upfront payment of approximately  $\leq 3,000,000$  ( $\leq 1,500,000$  in cash and  $\leq 1,500,000$  in uniQure shares), and will receive a further  $\leq 14,500,000$  in success-based milestone payments upon achieving certain clinical and regulatory targets. Upon a successful commercial launch of a developed product, the sellers will further receive a royalty payment of 0.5 % of the net product sales. The  $\leq 14,500,000$  in milestones is payable, at our sole discretion, in either cash or a variable number of uniQure shares, based on the then current share price.

### **Financial Operations Overview**

### Revenues

To date, we have not generated any revenues from royalties or product sales. We do not expect to generate royalty or product revenues prior to the commercial launch of Glybera by Chiesi. When and if Chiesi generates commercial sales of Glybera, we will record the gross amounts we receive from Chiesi as product revenues. We will record the related expenses, including third party royalties and related payments, as cost of goods sold.

During the three months ended March 31, 2015, we recognized collaboration revenues of  $\notin 0.9$  million associated with development activities that were reimbursable by Chiesi under our co-development agreement for hemophilia B. We expect to continue to recognize such collaboration revenues going forward, in accordance with our contractual agreements.

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During the three months ended March 31, 2015, we also recognized license revenues of  $\pounds$ 0.2 million. This amount reflects the amortization during the period of the non-refundable upfront payments we received from Chiesi under our collaboration agreements. The balance of  $\pounds$ 15.4 million of these license revenues will be recognized on a straight-line basis through the remaining period of the intellectual property protection of our manufacturing technologies, which is currently expected to be until September 2032.

The timing of our operating cash flows may vary from the revenue recognition of the related amounts, as we defer the recognition of some upfront payments, including the upfront payments under our Chiesi agreements, and recognize these as revenue when earned or over a defined period, while we treat other revenue, such as milestone payments or service fees, as earned when received. We expect our revenues to vary from quarter to quarter and year to year, depending upon, among other things, the commercial success of Glybera, our success in obtaining marketing approval for Glybera in the United States and additional countries, the structure and timing of milestone events, the number of milestones achieved, the level of revenues earned for ongoing development efforts, any new collaboration arrangements we may enter into and the terms we are able to negotiate with our collaborators. We currently intend to sell Glybera in the United States, if approved, ourselves, in which case we would recognize revenues in the full amount of the sales price. In addition, because LPLD is an orphan disease and we expect that the number of patients that will be treated with Glybera is relatively small, and because we currently expect that we will receive a one-time payment for a single patient treatment, we anticipate that revenues from Glybera may vary significantly from period to period. Further, because we currently anticipate that LPLD patients will require only a single administration with Glybera, we do not expect to earn recurring revenue from treated patients. We therefore believe that period-to-period comparisons should not be relied upon as indicative of our future revenues.

## Other Income

Our other income consists principally of government grants and subsidies that support our research efforts in defined research and development projects, which we refer to as grants. These grants generally provide for reimbursement of our approved expenses incurred as defined in various grants. We recognize grants when expenses are incurred in accordance with the terms and conditions of the grant and the collectability of the receivable is reasonably

assured. Because we have limited or no control over the timing of receipt of grants, the amount of other income varies from period to period and is not indicative of underlying trends in our operations.

We have received grants from the Dutch government and from the European Union. We have also participated in collaborations and consortia in which our collaborators and fellow consortium members have received grants from governmental authorities, which have enabled us to access preclinical and clinical data while minimizing the expenses we incur.

We have received a research and development subsidy from the Dutch government in the form of reimbursement of payroll taxes related to relevant employees. The amount we receive is tied directly to the number of employees and number of hours devoted to specified research and development programs, and therefore varies directly with the size of our workforce and direction of our research and development programs. We have no obligation to repay these amounts.

Some of the grants we have received are repayable under specified circumstances. In particular, we would be required to repay some grants if we successfully commercialize a supported program within a specified timeframe. None of the grants we have received to date relate to programs that we currently anticipate commercializing, other than the technical development loan in respect of Glybera, described under "Costs of Goods Sold" below. Accordingly, we do not currently expect that we will be required to repay any of these grants.

Other income also includes amounts we receive as payment or reimbursement for expenses of manufacturing and development of AMT-110 under our collaboration agreement with Institut Pasteur.

#### Cost of Goods Sold

Cost of goods sold includes the purchase price of raw materials, directly attributable labor costs and directly related charges by third party service providers, and the royalties and other related payments to third parties we must make under the license agreements covering various aspects of the technology underlying the composition and manufacture of Glybera.

We also include in cost of goods sold amounts that we are required to repay to the Dutch government in respect of a technical development loan that we received in the period from 2000 to 2005 to support the early development of Glybera. As of March 31, 2015, the total amount of principal and interest outstanding was  $\xi$ 5.9 million. Under the terms of this contingent commitment, we are required to make repayments based on the timing and amount of revenues we receive from product sales of Glybera. In connection with our receipt of upfront payments from Chiesi for the commercialization of Glybera, we repaid  $\xi$ 0.8 million of this loan in September 2013, which we recorded as cost of goods sold although no product sales occurred. No further payments will be made until sales of Glybera commence. We expect to pay to the Dutch government 20% of any gross amounts we

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receive from Chiesi in connection with sales of Glybera, as and when received, until the earlier of such time as the loan is repaid in full or December 31, 2019. Amounts that remain outstanding as of December 31, 2019, if any, would be forgiven. We have not recorded any liability for these amounts. To the extent we generate revenues from the sale of Glybera, we will recognize a liability and a corresponding charge to cost of goods sold in future periods.

Should we obtain marketing approval in the United States for Glybera, we expect that our costs of goods sold for sales of Glybera in the United States would be significantly lower than our costs of goods sold for sales of Glybera in the European Union due principally to the existence of lower royalty obligations on U.S. sales.

### **Research and Development Expenses**

Research and development expenses consist principally of expenses associated with employees, manufacturing facilities, clinical development, collaboration with third parties, license fees, laboratory consumables and depreciation.

During the period from 2006, when we received our first significant venture capital equity investment, to March 31, 2015, we incurred an aggregate of  $\pounds$ 140.5 million in research and development expenses. In addition, we began to capitalize our development expenses related to Glybera from March 21, 2013. We capitalized  $\pounds$ 7.9 million of such expenses through the period ended March 31, 2015, which we expect to begin amortizing once sales of Glybera commence, over the period through September 2032. We allocate our direct research and development expenses to our various programs on the basis of actual external expenses incurred in respect of each program and our allocation of time spent by our research and development team on each program. We do not allocate our overhead expenses to specific development programs. Our research and development expenses mainly relate to the following key programs:

- *Hemophilia B (HemB).* We have initiated a Phase I/II clinical trial of AMT-060 for the treatment of hemophilia B in the first quarter of 2015 in collaboration with Chiesi. Under our co-development agreement, we and Chiesi will each bear half of the development costs of this program.
- *CHF Program.* In the third quarter of 2014, we started to incur costs related to the pre-clinical development of product candidates targeting the S100A1 gene, the rights to which we obtained through our acquisition of InoCard in July 2014.
- *Glybera*. We are undertaking preparations for the EMA-mandated post-approval clinical trial and patient registry and preparing for the initiation of that study under an IND with the FDA in early 2016. We bear all of the costs of this program outside of the territories covered by the Chiesi agreement. Certain costs, including the patient registry for territories covered by the Chiesi agreement, will be shared equally with Chiesi.
- *Acute intermittent porphyria (AIP).* We have incurred costs related to the development and manufacture of clinical supplies of AMT-021 for the treatment of AIP provided to our collaborator, Digna Biotech, for its ongoing Phase I clinical trial in this indication.
- *CNS programs.* We have incurred costs related to the development and manufacture of clinical supplies of AMT-110 for the treatment of Sanfilippo B provided to our collaboration partner, Institut Pasteur, for its ongoing Phase I/II clinical trial. We also incur expenses related to the research and preclinical activities related to our other CNS programs.
- *Technology platform development and other research*. We incur significant research and development costs related to our gene delivery and manufacturing technology platform that are applicable across all of our programs, as well as our other research programs, including intellectual property expenses, depreciation expenses and facility costs. These costs are not allocated to specific projects.

Our research and development expenses may vary substantially from period to period based on the timing of our research and development activities, including regulatory approvals and enrollment of patients in clinical trials. We expect that our research and development expenses will increase significantly as we increase our staff, progress our Phase I/II study of AMT-060, conduct further clinical development of Glybera, advance the research and development of our other product candidates and commence manufacturing at our facility in Lexington, Massachusetts. The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or estimated costs of, or any cash inflows resulting from, the development of any of our product candidates. This is due to numerous risks and uncertainties associated with developing gene therapies, including the uncertainty of:

- · the scope, rate of progress and expense of our research and development activities;
- · clinical trial and early-stage results;
- the terms and timing of regulatory approvals;
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights; and
- our and our collaborators' ability to market, commercialize and achieve market acceptance for Glybera or any other product candidate that we may develop in the future.

A change in the outcome of any of these variables with respect to the development of Glybera or any other product

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candidate that we may develop could mean a significant change in the expenses and timing associated with the development of Glybera or such product candidate. For example, if the FDA or another regulatory authority were to require us to conduct preclinical and clinical studies for Glybera or any other product candidate beyond those which we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of our clinical development.

We have incurred significant expenses in the development of Glybera. Under applicable accounting principles, we capitalize development expenses upon receipt of marketing approval for a product candidate, provided that we have the technical, scientific and financial resources to complete the development and commercialization of the program. We received marketing approval from the European Commission for Glybera for a subset of LPLD patients in October 2012. Because we did not have sufficient financial resources at that time to complete the development of Glybera, including the postapproval activities required by the EMA prior to commercial launch, we did not capitalize the development expenses related to Glybera during the year ended December 31, 2012. Following our receipt of an additional €10.0 million in convertible debt financing in the first quarter of 2013, we determined that we had sufficient financial resources to complete these post-approval activities, and accordingly began to capitalize the related development expenses in the first quarter of 2013.

Over the period through 2016, we anticipate that we will incur external expenses related to the further development of Glybera, including implementation of the patient registry, initiation and conduct of the post-approval clinical trial and additional development work to seek FDA approval, of approximately €7.0 million; in addition, we will incur significant related employee expenses.

In addition, in connection with our collaboration and license agreement with 4D Molecular Therapeutics, we are incurring expenses to fund a joint research effort with 4D. Further, we granted options to purchase an aggregate of 609,744 of our ordinary shares to the owners of 4D who will be providing services to us in connection with that agreement. The fair value of these options will vest over a three-year future service period, and will have a significant impact on our expenses recognized. Finally, to the extent certain pre-clinical, clinical and regulatory milestones are met, we will make milestone payments to 4D.

#### Selling, General and Administrative Expenses

Our selling, general and administrative expenses have consisted to date principally of employee, office, consultancy and other administrative expenses. We expect that our selling, general and administrative expenses will increase significantly in the future as our business expands and we add personnel and infrastructure, and as we commence manufacturing operations in our facility in Lexington, Massachusetts. We also incur additional expenses associated with operating as a public company, including expenses for additional personnel, additional legal, accounting and audit fees, directors' and officers' liability insurance premiums and expenses related to investor relations. In future periods, we will include in selling, general and administrative expenses our sales expenses related to the commercialization of Glybera, including our market access efforts, as well as the costs related to the sales and marketing efforts we intend to undertake in the United States in advance of potential marketing approval for Glybera from the FDA.

#### Other Gains / Losses-Net

Other gains / losses—net consist of foreign exchange losses that do not relate to borrowings. We are exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. dollar and, to a lesser extent, the British pound, as we acquire certain materials and pay for certain licenses and other services in these two currencies. We have not established any formal practice to manage the foreign exchange risk against our functional currency.

### **Finance** Income

Our finance income consists of interest income earned on our cash and cash equivalents and gains on our derivative instruments, described below. We deposit our cash and cash equivalents primarily in savings and deposit accounts with original maturities of three months or less. Savings and deposit accounts have historically generated only minimal interest income.

We have entered into various financing arrangements with our investors, including convertible notes issued in 2009 and converted into ordinary shares in April 2012, and further convertible notes issued in 2012 and 2013, which were converted into ordinary shares in July 2013. Each of the convertible notes consists of a debt element and an embedded financial derivative element. Derivatives are initially recognized at fair value on the date a derivative contract is entered into and are subsequently measured at fair value through profit and loss. The resulting gain is recognized in the Consolidated Income Statement and accounted for as finance income.

## Finance Expense

Finance expenses in 2014 and 2015 consisted primarily of interest due on our Hercules venture debt loan, losses on the fair value measurements of our derivative instruments, and, to a lesser extent, the interest component of finance leases.

## **Results of Operations**

## Comparison of the three months ended March 31, 2014 and 2015

|  | THREE MONTH<br>MARCH 3 |          |             |  |
|--|------------------------|----------|-------------|--|
|  | 2014                   | 2015     | Change      |  |
|  | (€ in thousands)       |          | %           |  |
| License revenues                             | 220                    | 221      | 0%          |  |
| Collaboration revenues                       | 950                    | 886      | -7%         |  |
| Total revenues                               | 1,170                  | 1,107    |             |  |
| Cost of goods sold                           |                        | _        |             |  |
| Other income                                 | 238                    | 206      | -13%        |  |
| Research and development expenses            | (6,218)                | (10,106) | 63%         |  |
| Selling, general and administrative expenses | (2,268)                | (4,159)  | 83%         |  |
| Other gains / losses, net                    | (519)                  | 4,245    | -918%       |  |
| Total operating costs                        | (8,767)                | (9,814)  | 12%         |  |
| Operating result                             | (7,597)                | (8,707)  | 15%         |  |
| Finance income                               | 27                     | 19       | -30%        |  |
| Finance expense                              | (259)                  | (3,950)  | 1425%       |  |
| Net loss                                     | (7,829)                | (12,638) | <b>61</b> % |  |

#### Revenues

License revenues for the three months ended March 31, 2015 were €0.22 million, in line with the €0.22 million for the three months ended March 31, 2014.

Collaboration revenues for the three months ended March 31, 2015 were €0.89 million, a 7% decrease from the €0.95 million for the three months ended March 31, 2014.

## **Other Income**

Other income for the three months ended March 31, 2015, was  $\in 0.21$  million, a 13% decrease from the  $\in 0.24$  million recognized for the three months ended March 31, 2014. This change reflected a slight increase in research activities effort related government grants received.

### **Research and Development Expenses**

Research and development expenses for the three months ended March 31, 2015 were  $\leq 10.1$  million, a 63% increase from the  $\leq 6.2$  million incurred for the three months ended March 31, 2014. These increases are mainly due to the initiation of our Phase I/II clinical study of AMT-060 in hemophilia, additional development and clinical activities required to support the planned commercial launch of Glybera, the continued progression of uniQure's other programs through later stage research and clinical development and increased activity in our U.S. facility, which became only operational in the second half of 2014.

Glybera-related raw materials that cannot be used for commercial purposes are expensed; Glybera-related materials, including raw materials, workin-progress and finished goods, that are expected to be used for commercial purposes are recorded as inventory on the balance sheet and are not accounted for within research and development expenses.

### Selling, General and Administrative Expenses

Selling, general and administrative expenses for the three months ended March 31, 2015 were  $\leq 4.2$  million, a 83% increase from the  $\leq 2.3$  million incurred for the three months ended March 31, 2014. These increases are primarily due to expenses related to consultants (commercial and administrative) and professional fees.

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### Other losses—Net

Other gains / losses—net for the three months ended March 31, 2015 were a gain of  $\notin$ 4.2 million, compared with a loss of  $\notin$ 0.52 million for the three months ended March 31, 2014. This increase reflects changes in the foreign exchange rate between the euro and the U.S. dollar.

#### Finance Income

Finance income for the three months ended March 31, 2015 was  $\notin 0.02$  million, compared with  $\notin 0.03$  million for the three months ended March 31, 2014. This reflects the increased interest income associated with our higher average cash balance in 2014 compared to 2013.

#### **Finance** Expense

Finance expense for the three months ended March 31, 2015 was &4.0 million, compared with &0.3 million for the three months ended March 31, 2014. This increase related to an increase in interest payments following the amended and increased venture debt loan with Hercules Technology Growth Corporation as well as the expenses related to the adjustment to Fair Market Value from our outstanding warrants.

### Liquidity and Capital Resources

In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. From our first institutional venture capital financing in 2006 until our initial public in February 2014, we funded our operations primarily through private and public placements of equity securities, and convertible and other debt securities, in the aggregate amount of  $\pounds$ 204.1 million (\$269.8 million). During the period as indicated above, we also received total other income, consisting principally of government grants and subsidies, of  $\pounds$ 6.5 million, and total nonrefundable collaboration funding of  $\pounds$ 17.0 million, and \$20.0 million ( $\pounds$ 14.6 million) in venture debt financing.

On February 5, 2014 the Company successfully completed its initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91.8 million ( $\in 67.3$  million) and net proceeds of \$85.4 million ( $\in 62.6$  million) after commissions but before expenses.

On April 6, 2015, the Company entered into agreements with BMS, the financial terms of which consist of guaranteed, near-term payments to the Company of \$103 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the strategic collaboration occurred on May 21, 2015. An additional \$15 million payment is to be received following the selection of three additional collaboration targets, in addition to the \$100A1 program, within three months of the closing. In addition, an initial equity investment in the Company will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or about \$38 million in total. This investment is expected to be completed on June 12, 2015. BMS is also obligated to make an additional equity investment in the Company for a number of shares outstanding following such issuance by December 31, 2015 and will be granted two warrants to acquire up to an additional 10% equity interest, at a premium to market, based on additional targets being introduced into the collaboration. The Company will also be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead \$100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration, as well as net sales based milestone payments and tiered single to double-digit royalties on product sales.

On April 15, 2015 the Company announced the closing of its follow-on public offering of 3,000,000 ordinary shares at price to the public of \$29.50 per ordinary share. After deducting underwriting discounts but before share issuance expenses, the net proceeds of the follow-on public offering were \$83.2 million ( $\in$ 78.5 million).

On June 10, 2015, the shareholders of uniQure approved the equity component of the BMS collaboration, and the Company will issue an initial tranche of 1,112,300 ordinary shares to BMS in June for aggregate consideration of \$37.6 million.

As of March 31, 2015, we had cash and cash equivalents of €43.2 million. To date, we have not generated any revenues from royalties or product sales. We do not expect to generate royalty or revenues from product sales prior to the commercial launch of Glybera by Chiesi.

## Cash flows

Our cash and cash equivalents as of March 31, 2015 were €43.2 million. The table below summarizes our Consolidated Cash Flow

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data for the unaudited three month periods ended March 31, 2014 and 2015:

|  |         | Three months ended<br>March 31, |  |  |
|--|---------|---------------------------------|--|--|
| (€ in thousands)                                   | 2014    | 2015                            |  |  |
| Cash (used in) / generated by operating activities | (5,069) | (12,046)                        |  |  |
| Cash used in investing activities                  | (3,126) | (2,007)                         |  |  |
| Cash provided by financing activities              | 61,915  | 107                             |  |  |

## Net Cash (Used in)/Generated by Operating Activities

Net cash used in operating activities was  $\leq 12.0$  million in the three months ended March 31, 2015, a 138% increase from the  $\leq 5.1$  million used in the three months ended March 31, 2014. The change principally reflected the higher net loss from operating activities, driven by increased research and development activities and a higher level of activity and expense in our U.S. facility, which became only operational in the second half of 2014.

## Net Cash Used in Investing Activities

Net cash used in investing activities was €2.0 million in the three months ended March 31, 2015, a 36% reduction from the €3.1 million in the three months ended March 31, 2014. Investing activities in both periods reflect the build out of our U.S. facility.

## Net Cash Generated from Financing Activities

Net cash generated from financing activities was 0.1 million in the three months ended March 31, 2015, compared to the 0.1 million in the three months ended March 31, 2014. The 2014 amount reflected the receipt of 0.1 million, after commissions and expenses, in connection with our initial public offering in February 2014. The 2015 amount is related to the issuance of shares following the exercise of options.

## **Cash and Funding Sources**

The table below summarizes our sources of financing for the three months ended March 31, 2014 and 2015.

| (€ in thousands)                  | Equity<br>Capital |
|-----------------------------------|-------------------|
| Three months ended March 31, 2015 | 147               |
| Three months ended March 31, 2014 | 61,953            |

- Our sources of financing in the three months ended March 31, 2015 were related to the issuance and sale of 336,672 ordinary shares upon the exercise of options granted to employees and other parties
- As of March 31, 2015, we had debt of €18.4 million, which consisted solely of amounts outstanding under the Hercules Agreement.

## Funding Requirements

We believe our cash and cash equivalents as at March 31, 2015, when taken together with additional funds raised since that date following the closing of our collaboration with Bristol-Myers Squibb and the follow on public offering of our ordinary shares, will enable us to fund our operating expenses, including our debt repayment obligations as they become due, and capital expenditure requirements, for the next twelve months. For further disclosure please refer to Note 1 of the unaudited financial statements for the period ended March 31, 2015, for a description of the BMS Collaboration and the follow on public offering. Our future capital requirements will depend on many factors, including:

- the potential to receive future consideration pursuant to our collaboration with BMS, which is largely contingent on achieving certain research, development, regulatory and sales milestones;
- the commercial success of Glybera, including the timing and amount of revenues generated, as well as our cost of goods sold;
- our collaboration agreements remaining in effect, our ability to obtain research and development funding and achieve milestones under these agreements and our ability to enter into other such new arrangements in the future;

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- the progress and results of our current and planned clinical trials, including for Glybera and AMT-060 for hemophilia B, as well as those of our collaborators;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for S100A1 in congestive heart failure and our additional product candidates;
- the number and development requirements of other product candidates that we pursue;
- the cost, timing and outcome of regulatory review of our product candidates, particularly for approval of Glybera in the United States;
  the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and
- the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and distribution, for Glybera and any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we receive marketing approval in the future;
- expenses in connection with our collaboration with 4D Molecular Therapeutics;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the repayments of the principal amount of our venture debt loan with Hercules Technology Growth Capital, that contractually will start in January 2016 and will run through June 2018;
- the extent to which we acquire or in-license other products or technologies; and
- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility.

We have no committed sources of additional financing, other than our collaboration agreements with BMS and Chiesi. Until such time, if ever, as we can generate substantial product revenues from sales of Glybera by Chiesi or otherwise, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution and licensing arrangements. We are subject to covenants under the Hercules Agreement, and may become subject to covenants under any future indebtedness that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business. In addition, our pledge of assets as collateral to secure our obligations under the Hercules Agreement may limit our ability to obtain debt financing. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

#### **Capital Expenditures**

The following table sets forth our capital expenditures for the three months ended March 31, 2014 and 2015:

|  |         | Three months ended<br>March 31 |  |  |
|--|---------|--------------------------------|--|--|
| (€ in thousands)                             | 2014    | 2015                           |  |  |
| Investments in property, plant and equipment | (2,025) | (1,569)                        |  |  |
| Investments in intangible assets             | (1,148) | (538)                          |  |  |
| Total  | (3,173) | (2,107)                        |  |  |

In the first quarter of 2015 we nearly completed the build-out a 53,000 square foot leased manufacturing facility in Lexington, Massachusetts. The total construction costs amount to approximately \$16.8 million, of which the landlord has paid \$7.3 million in landlord improvements. In addition, we anticipate the total investment in property, plant and equipment to be approximately \$8.2 million. As of March 31, 2015, we had capitalized \$24.2 million and had contractual commitments of a further \$0.4 million. In addition, we provided a landlord deposit of \$1.2 million.

The investments in Intangible Assets relate to the capitalization of licenses and the ongoing capitalization of Glybera-related development costs.

The table below sets forth our contractual obligations and commercial commitments as of March 31, 2015 that are expected to have

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an impact on liquidity and cash flows in future periods.

| (€ in thousands)                       | LESS THAN<br>1 YEAR | BETWEEN 1<br>AND 2 YEARS | BETWEEN 2<br>AND 5 YEARS | OVER 5<br>YEARS | TOTAL  |
|--|---------------------|--------------------------|--------------------------|-----------------|--------|
| License maintenance obligations (1)(2) | 337                 | _                        | _                        | _               | 337    |
| Debt obligations                       | 3,161               | 7,773                    | 9,608                    | —               | 20,542 |
| Operating lease obligations            | 2,097               | 1,882                    | 5,150                    | 7,717           | 16,846 |
| Finance lease obligations              | 171                 | 90                       |                          | —               | 261    |
| Construction commitment US Facility    | 345                 |                          | _                        | —               | 345    |
| Total                                  | 6,111               | 9,745                    | 14,758                   | 7,717           | 38,331 |

(1) Annual license maintenance payments will be no longer payable following the expiration of the license payment obligations. Thereafter, we have a fully paid-up license.

(2) Amounts are paid annually in advance; to the extent that we could terminate the agreement prior to the date of the next maintenance payment, these maintenance fees are not included within the research commitments detailed in the notes to the Financial Statements.

The table above does not include:

- Payments we may be obligated to make under our license or collaboration agreements, other than fixed periodic maintenance costs. Such additional payment obligations may be material.
- Our obligations to repay the Dutch technical development loan described above.
- Our obligations under the collaboration and license agreement with 4D Molecular Therapeutics, entered into in January 2014, to fund research and development activities at a cost of approximately \$3.0 million in aggregate over the first three years and approximately \$200,000 of licenses fees during the first year.

## Hercules Loan and Security Agreements

We are party to a Loan and Security Agreement entered into with Hercules on June 13, 2013. Under the Loan and Security Agreement, we borrowed  $10.0 \text{ million} \in 7.4 \text{ million}$  from Hercules, bearing interest at a variable rate of the greater of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%.

On June 26, 2014 we entered into an amended and restated loan agreement (which amends and replaces the original loan agreement) of 20,000,000 (then 14,600,000), presented net of expenses for facility charges of 1.00% plus expenses related to legal counsel. The additional amount of 10,000,000 (7,344,000) was received net of expenses of 218,000 (160,000). The net cash inflow was 9,782,000 (7,184,000). The total loan commitment is 20,000,000 with an interest rate of 10.25%. Also included are two back-end fees of 345,000 and 250,000, due October 2016 and June 2018 respectively. The interest-only period is 18 months. We are required to repay the loan in monthly principal installments from January 2016 through June 2018. As the terms of the amended loan agreement changed significantly compared to the original loan agreement (maturity date, interest rate, payback schedule), the Company fully amortized the unamortized transaction costs at issue, resulting in an extra amortization charge through profit and loss in 2014 of 193,000 (141,000).

The amended Loan and Security Agreement also provides for payment of a maturity charge, the amount of which was reduced in exchange for the issuance to Hercules, on September 24, 2013, of 37,174 warrants, at an exercise price of  $\leq 10.10$  per share. The warrant included in the Loan and Security Agreement is not closely related to the host contract and therefore has been split and accounted-for separately as a financial derivative measured at fair value though profit or loss. The fair value of this derivative as of March 31, 2015 was  $\leq 490,000$  compared to  $\leq 216,000$  on March 31, 2014.

The borrowings under the Loan and Security Agreement were classified as non-current borrowings of  $\leq 17.1$  million and as current borrowings of  $\leq 1.4$  million, net of expenses, as of March 31, 2015. For the three month period ended March 31, 2015, we recorded  $\leq 0.5$  million as finance expenses in relation to the Loan and Security Agreement, compared to  $\leq 0.3$  million for the same period in 2014.

The exchange result on the borrowings under the Loan and Security Agreement amounts to €2.0 million.

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