

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **March 14, 2018**

uniQure N.V.

(Exact Name of Registrant as Specified in Charter)

The Netherlands
(State or Other
Jurisdiction of Incorporation)

001-36294
(Commission
File Number)

N/A
(IRS Employer
Identification No.)

**Paasheuvelweg 25a,
1105 BP Amsterdam, The Netherlands**
(Address of Principal Executive Offices)

N/A
(Zip Code)

Registrant's telephone number, including area code: **+31-20-566-7394**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒ x

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒ x

Item 2.02. Results of Operations and Financial Condition.

We issued a press release on March 14, 2018 announcing uniQure N.V.'s (the "Company's") financial results for the full year ended December 31, 2017 and providing an update on the Company's progress. A copy of this press release is attached hereto as Exhibit 99.1.

The information contained in this Current Report on Form 8-K, including the Exhibit attached hereto, is being furnished under Item 2.02 and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Securities Exchange Act of 1934, except as shall be expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits

99.1 Press release dated March 14, 2018, relating to the Company's financial results for the full year ended December 31, 2017 (furnished only).

SIGNATURE

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 hereunto duly authorized.

UNIQUE N.V.

Date: March 14, 2018

By: /s/ MATTHEW KAPUSTA

Name: Matthew Kapusta

Title: Chief Executive Officer and Interim Chief Financial Officer

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EXHIBIT INDEX

Exhibit No.	Description
99.1	Press release dated March 14, 2018, relating to the Company's financial results for the full year ended December 31, 2017 (furnished only).

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uniQure Announces 2017 Financial Results and Recent Company Progress

~ On Track to Begin Pivotal Study of AMT-061 in Hemophilia B in Q3 2018

~ Submitted IND Amendment for AMT-061 Dose Confirmation Study, with Topline Data Expected by End of 2018

~ Expects IND Submission for AMT-130 in Huntington's Disease in the Second Half of 2018

~ \$159 million of cash and cash equivalents as of December 31, 2017

Lexington, MA and Amsterdam, the Netherlands, March 14, 2018 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today reported its financial results for 2017 and highlighted recent progress across its business.

"We ended 2017 with significant momentum across all of our gene therapy programs, and a strong cash position to fund our business into 2020," stated Matthew Kapusta, chief executive officer of uniQure. "We remain highly focused on initiating our pivotal study for AMT-061 in hemophilia B and advancing AMT-130 into a Phase I/II study in Huntington's disease. We look forward to providing updates on our progress throughout the year, including the presentation of top-line clinical data on AMT-061 by year-end."

Recent Company Progress:

- *Advancing AMT-061 for the treatment of hemophilia B into a pivotal trial*
 - In the first quarter of 2018, the Company submitted an Investigational New Drug (IND) amendment to the U.S. Food and Drug Administration (FDA) supporting its planned AMT-061 dose-confirmation study. Extensive data including comparability, manufacturing capability and non-clinical safety and bioequivalence were included in the submission. The Company anticipates initiating the treatment of patients early in the third quarter of 2018 at a single dose of 2 x 10¹³ gc/kg. Top line data from these patients are expected to be presented by the end of the year.
 - The process of site selection for the pivotal phase III study has been initiated and reviews by institutional review boards (IRB) are underway. The Company expects to begin patient enrollment in the lead-in phase of the pivotal study in the third quarter of 2018.
 - In the first quarter of 2018, the Company completed the full comparability analysis for AMT-061, which the Company believes continues to support the comparability between AMT-060 and AMT-061.
 - The Company has completed the manufacturing and full quality release of product for use in the dose confirmation study. The product was produced in the Company's state-of-the-art manufacturing facility in Lexington, Massachusetts.
 - The European Medicines Agency (EMA) issued a positive opinion on Orphan Drug Designation for AMT-061 in hemophilia B.
- *Advancing AMT-130 for the treatment of Huntington's disease into a Phase I/II study*
 - Preclinical data presented at the 13th Annual Huntington's Disease Therapeutics Conference in Palm Springs, CA demonstrated that knocking down human mutant huntingtin (mHTT) by AMT-130 was safe and well-tolerated in both the deep structure of the striatum and the cortex when evaluated in a humanized mouse model of Huntington's disease. A single administration of AMT-130 resulted in sustained dose-dependent distribution of AAV5, consistent with previous experience, strongly corresponding with human mHTT lowering in this model. Expression of total mHTT was significantly reduced by up to 87% in the striatum and up to 62% in the cortex at seven months post treatment, resulting in improvements of select behavioral and neuropathological Huntington's disease phenotypes. Treatment was well tolerated with no adverse events, including no psychiatric or cognitive defects.
 - A GLP-safety and toxicology study in non-human primates is ongoing and expected to conclude mid-year. Data from this study will be submitted in support of the IND, which is expected to be submitted to the FDA in the second half of 2018.
 - In January, the Company announced that it had received Orphan Medicinal Product Designation (OMDP) for AMT-130 in Huntington's disease by the EMA. OMPD offers product market exclusivity for ten years in the European Union following regulatory approval, along with tax and financial incentives for companies developing medicines for such orphan indications.
- *Continuing research collaboration with Bristol Myers-Squibb, including AMT-126 for the treatment of congestive heart failure*
 - Under the collaboration with Bristol-Myers Squibb, a preclinical study of AMT-126 to assess heart function in a diseased pig model was initiated in January 2018. Pending data from this study, which is expected later this year, the Company and Bristol-Myers Squibb plan to initiate a safety and toxicology study to enable an IND submission.
- *Adding talent to leadership team*

- On March 12, the Company appointed David Cerveny, J.D., as Chief Legal Officer. Prior to joining uniQure, Mr. Cerveny was Chief Legal Officer, General Counsel and Secretary at ConforMIS, Inc., a publicly traded medical technology company. Prior to this, he was Chief Intellectual Property Counsel at Palomar Medical Technologies, Inc., which was subsequently acquired by Cynosure. Earlier in his career, Mr. Cerveny was a partner at Hale & Dorr LLP (now WilmerHale). Mr. Cerveny earned a Bachelor of Science degree in biomedical engineering from Marquette University and a Juris Doctor degree from Boston College Law School.

Upcoming Anticipated Milestones

- Initiate dosing of patients in the dose confirmation study of AMT-061, data from which will be available by the end of 2018
- Initiate patient enrollment in the lead-in phase of the pivotal study of AMT-061 in the third quarter of 2018
- Completion of the GLP-safety and toxicology study of AMT-130 and submission of the IND in Huntington's disease in the second half of 2018
- Completion of a preclinical therapeutic heart function study of AMT-126 in congestive heart failure in the second half of 2018
- Expansion of the Company's early-stage research pipeline

Financial Highlights

Cash Position: As of December 31, 2017, the Company held cash and cash equivalents of \$159.4 million, compared to \$132.5 million as of December 31, 2016. In October 2017, the Company raised net proceeds of \$85.3 million in an underwritten public offering of ordinary shares. The Company currently expects cash and cash equivalents will be sufficient to fund operations into early 2020.

Revenues: Revenue for the year ended December 31, 2017 was \$13.1 million, compared to \$25.1 million for the year ended December 31, 2016. Collaboration revenue for the year ended December 31, 2017 was \$9.0 million, compared to \$20.2 million for the year ended December 31, 2016. The decrease in collaboration revenue was primarily due to the termination of the Chiesi co-development agreement in July 2017, as well as nonrecurring revenue recognized in the prior year period associated with the production of AMT-126 product supplies.

R&D Expenses: Research and development expenses were \$72.2 million for the year ended December 31, 2017, compared to \$72.5 million for the year ended December 31, 2016. During the year ended December 31, 2017, we recorded expenses related to an increase in the fair market value of the contingent consideration owed to the sellers of the InoCard business, which was offset by cost savings associated with the consolidation of manufacturing activities into our Lexington, Massachusetts facility.

SG&A Expenses: Selling, general and administrative expenses were \$24.6 million for the year ended December 31, 2017, compared to \$26.0 million for the year ended December 31, 2016. The decrease in SG&A expenses was primarily due to certain one-time costs incurred in 2016 related to the settlement of our arbitration proceedings with Extera and the conversion of our financial reporting to U.S. GAAP. This reduction was offset, in part, by an increase in share-based compensation costs during the year ended December 31, 2017 compared to the prior year.

Other income: Other income was \$15.4 million for the year ended December 31, 2017, compared to \$1.5 million for the year ended December 31, 2016. The current year period includes the full amortization of the outstanding deferred revenue of \$13.8 million following the termination of the Company's collaboration with Chiesi in July 2017.

Net Loss: The net loss was \$79.3 million, or \$2.94 per share, for the year ended December 31, compared to \$73.4 million, or \$2.93 per share, for the year ended December 31, 2016.

About uniQure

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

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, our upcoming anticipated milestones, the development of our gene therapy product candidates, the transition to our AMT-061 product candidate, the success of our collaborations and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with our and our collaborators' clinical development activities, collaboration arrangements, corporate reorganizations and strategic shifts, regulatory

oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's Annual Report on Form 10-K filed on March 14, 2018. 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 CONSOLIDATED BALANCE SHEETS

	December 31, 2017	December 31, 2016
	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	\$ 159,371	\$ 132,496
Accounts receivables and accrued income	1,586	9,180
Prepaid assets and other current assets	1,826	2,270
Total current assets	162,783	143,946
Non-current assets		
Property, plant and equipment, net	34,281	35,702
Intangible assets and goodwill	10,100	8,789
Other non-current assets	2,480	1,828
Total non-current assets	46,861	46,319
Total assets	\$ 209,644	\$ 190,265
Current liabilities		
Accounts payable	\$ 2,908	\$ 5,524
Accrued expenses and other current liabilities	8,838	9,766
Current portion of long-term debt	1,050	605
Current portion of deferred rent	737	684
Current portion of deferred revenue	4,613	6,142
Current portion of contingent consideration	1,084	—
Total current liabilities	19,230	22,721
Non-current liabilities		
Long-term debt, net of current portion	19,741	19,631
Deferred rent, net of current portion	9,114	6,781
Deferred revenue, net of current portion	67,408	75,612
Contingent consideration, net of current portion	2,880	1,838
Derivative financial instruments related party	1,298	51
Other non-current liabilities	614	—
Total non-current liabilities	101,055	103,913
Total liabilities	120,285	126,634
Total shareholders' equity	89,359	63,631
Total liabilities and shareholders' equity	\$ 209,644	\$ 190,265

uniQure N.V.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Years ended December 31,		
	2017	2016	2015
	in thousands, except share and per share amounts		
Total revenues	\$ 13,107	\$ 25,098	\$ 10,578
Operating expenses:			
Research and development expenses	(72,172)	(72,510)	(59,125)
Selling, general and administrative expenses	(24,635)	(25,999)	(23,383)
Total operating expenses	(96,807)	(98,509)	(82,508)
Other income	15,430	1,465	779
Other expense	(3,073)	—	—
Loss from operations	(71,343)	(71,946)	(71,151)
Non operating items, net	(8,116)	(283)	(12,111)
Loss before income tax expense	(79,459)	(72,229)	(83,262)
Income tax benefit / (expense)	199	(1,145)	1,179
Net loss	\$ (79,260)	\$ (73,374)	\$ (82,083)

Basic and diluted net loss per common share	\$	(2.94)	\$	(2.93)	\$	(3.72)
Weighted average shares used in computing basic and diluted net loss per common share		26,984,183		25,036,465		22,082,345