uniQure N.V.
(Exact name of Registrant as specified in its charter)
The Netherlands
(Jurisdiction of incorporation or organization)
Paasheuvelweg 25a,
1105 BP Amsterdam, The Netherlands
(Address of principal executive offices) (Zip Code)
+31-20-240-6000
(Registrant’s telephone number, including area code)

Title of Each Class Trading Symbol(s) Name of Each Exchange on Which Registered
Ordinary shares, par value €0.05 per share QURE The Nasdaq Stock Market LLC (The Nasdaq Global Select Market)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☒ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☒ Accelerated filer ☐ Non-accelerated filer ☐ Smaller reporting company ☐ Emerging growth company ☐

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.

☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management’s assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act.) Yes ☐ No ☒

The aggregate market value of the voting and non-voting ordinary shares held by non-affiliates of the registrant as of June 30, 2020 was $2,002.7 million, based on the closing price reported as of June 30, 2020 on the NASDAQ Global Select Market.

As of February 25, 2021, the registrant had 44,993,987 ordinary shares, par value €0.05, outstanding.

The documents incorporated by reference are as follows:

 Portions of the registrant’s definitive Proxy Statement for its 2021 Annual Meeting of Shareholders to be filed with the Securities and Exchange Commission no later than April 30, 2021 and to be delivered to shareholders in connection with the 2021 Annual Meeting of Shareholders, are herein incorporated by reference in Part III of this Annual Report on Form 10-K.
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SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains “forward-looking statements” as defined under federal securities laws. Forward-looking statements are based on our current expectations of future events and many of these statements can be identified using terminology such as “believes,” “expects,” “anticipates,” “plans,” “may,” “will,” “projects,” “continues,” “estimates,” “potential,” “opportunity” and similar expressions. These forward-looking statements, which include, but are not limited to, statements related to the COVID-19 coronavirus pandemic, our collaboration and license agreement with CSL Behring LLC and the timing of the completion of the transactions contemplated thereby, our beliefs about our competitive advantage and the capabilities of our manufacturing facility, our cash runway, the advancement of our clinical trials, our intellectual property portfolio, and the impact of regulatory actions on our regulatory submission timelines, may be found in Part I, Item 1 “Business,” Part 1, Item 1A “Risk Factors,” Part II, Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other sections of this Annual Report on Form 10-K.

Forward-looking statements are only predictions based on management’s current views and assumptions and involve risks and uncertainties, and actual results could differ materially from those projected or implied. The most significant factors known to us that could materially adversely affect our business, operations, industry, financial position or future financial performance include those discussed in Part I, Item 1A “Risk Factors,” as well as those discussed in Part II, Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Annual Report on Form 10-K, as well as other factors which may be identified from time to time in our other filings with the Securities and Exchange Commission ("SEC"), or in the documents where such forward-looking statements appear. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. Our actual results or experience could differ significantly from those anticipated in the forward-looking statements and from historical results, due to the risks and uncertainties described in this Annual Report on Form 10-K including in “Part I, Item 1A. “Risk Factors,” as well as others that we may consider immaterial or do not anticipate at this time. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future or may file or furnish with the SEC. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Annual Report on Form 10-K to reflect later events or circumstances or to reflect the occurrence of unanticipated events. All forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements.

In addition, with respect to all our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.
Summary Risk Factors

An investment in our ordinary shares involves significant risks. You should carefully consider the information set forth under “Risk Factors” before deciding to invest in our ordinary shares. The following is a summary of the principal risks associated with an investment in our ordinary shares:

- We and CSL Behring may be unable to close the transaction contemplated by the CSL Behring Agreement, and any delay in closing the transaction could diminish the anticipated benefits of the transaction or result in increased costs. Failure to close the transaction could adversely impact the market price of our ordinary shares as well as our business and operating results, cash flows and results of operations.

- We may encounter substantial delays in, and impediments to the progress of our clinical trials or fail to demonstrate the safety and efficacy of our product candidates, and our clinical trials for AMT-061 are currently on clinical hold and could remain on clinical hold indefinitely.

- Our business and operations have been, and may continue to be, materially and adversely affected by the ongoing COVID-19 pandemic.

- We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates.

- We may not be successful in our efforts to in-license or acquire product candidates that align with our research and development strategy.

- Our manufacturing facility is subject to significant government regulations and approvals. If we fail to comply with these regulations or to maintain these approvals our business could be materially harmed.

- Our resources might be adversely affected if we are unable to meet our product development and supply needs and obligations, including our ability to complete the validation of our existing manufacturing processes as well as to develop larger scale manufacturing processes, which could adversely affect our ability to sufficiently meet our future production needs or regulatory filing timelines.

- Our resources might be adversely affected if we are unable to meet our product supply needs and obligations.

- We cannot predict when or if we will obtain marketing approval to commercialize a product candidate.

- We are exposed to a number of external factors such as competition, insurance coverage of and pricing and reimbursement for our product candidates that may adversely affect our product revenue and that may cause our business to suffer.

- We rely on licenses of intellectual property from third parties, and such licenses may not provide adequate rights or may not be available in the future on commercially reasonable terms or at all, and our licensors may be unable to obtain and maintain patent protection for the technology or products that we license from them.

- If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our ability to successfully commercialize our products may be impaired.

- Our reliance on third parties may require us to share our trade secrets, which could increase the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

- We will likely need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may force us to delay, limit or terminate our product development efforts or other operations which could have a material adverse effect on our business, financial condition, results of operations, and cash flows.

- Our relationships with customers and third-party payers will be subject to applicable anti-kickback, anti-bribery, fraud and abuse and other laws and regulations, which, if we are found in violation thereof, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

- We are subject to laws governing data protection in the different jurisdictions in which we operate. The implementation of such data protection regimes is complex, and should we fail to fully comply, we may be subject to penalties that may have an adverse effect on our business, financial condition, and results of operations.
• Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

• If we fail to maintain an effective system of internal controls, we may be unable to accurately report our results of operations or prevent fraud or fail to meet our reporting obligations, and investor confidence and the market price of our ordinary shares may be materially and adversely affected.
Part I

Unless the context requires otherwise, references in this report to “uniQure,” “Company,” “we,” “us” and “our” and similar designations refer to uniQure N.V. and our subsidiaries.

Item 1. Business.

Overview

We are a leader in the field of gene therapy, seeking to develop single treatments with potentially curative results for patients suffering from genetic and other devastating diseases. We are advancing a focused pipeline of innovative gene therapies, including product candidates for the treatment of hemophilia B, which we intend to license to CSL Behring pursuant to the CSL Behring Agreement (as defined below), and Huntington’s disease. We believe our validated technology platform and manufacturing capabilities provide us distinct competitive advantages, including the potential to reduce development risk, cost, and time to market. We produce our Adeno-associated virus (“AAV”) -based gene therapies in our own facilities with a proprietary, commercial-scale, current good manufacturing practices (“cGMP”)-compliant, manufacturing process. We believe our Lexington, Massachusetts-based facility is one of the world’s most versatile gene therapy manufacturing facilities.

Key events

CSL Behring commercialization and license agreement

On June 24, 2020, uniQure biopharma B.V., a wholly-owned subsidiary of uniQure N.V., entered into a commercialization and license agreement (as amended, the “CSL Behring Agreement”) with CSL Behring LLC (“CSL Behring”) pursuant to which CSL Behring will receive exclusive global rights to etranacogene dezaparvovec, our investigational gene therapy for patients with hemophilia B (the “Product”).

Under the terms of the CSL Behring Agreement, we will receive a $450.0 million upfront cash payment upon the closing of the CSL Behring Agreement and be eligible to receive up to $1.6 billion in additional payments based on regulatory and commercial milestones. The CSL Behring agreement also provides that we will be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percent of net sales of the Product based on sales thresholds.

Pursuant to the CSL Behring Agreement, we will be responsible for the completion of the HOPE-B clinical trial, manufacturing process validation, and the manufacturing supply of the Product until such time that these capabilities may be transferred to CSL Behring or its designated contract manufacturing organization. Concurrently with the execution of the CSL Behring Agreement, we and CSL Behring entered into a development and commercial supply agreement, pursuant to which, among other things, we will supply the Product to CSL Behring at an agreed-upon price. Clinical development and regulatory activities performed by us pursuant to the CSL Behring Agreement will be reimbursed by CSL Behring. CSL Behring will be responsible for global regulatory submissions and commercialization requirements for the Product.

Other than under the CSL Behring Agreement, neither we nor CSL Behring may perform any clinical trials, with the exception of trials required to extend the label or gain marketing authorization outside the United States or the European Union, for any gene therapy product, gene-editing product, or any other product comprising an AAV vector to conduct nucleotide transfer (including deoxyribonucleic acid (“DNA”) and ribonucleic acid (“RNA”)) for the treatment, prevention, or cure of hemophilia B for a period commencing on June 24, 2020 and continuing for a period of four years following the first commercial sale of the Product in the United States, and neither we nor CSL Behring may commercialize such a product for a period commencing as of June 24, 2020 and continuing for a period of seven years following the first commercial sale of the Product in the United States. This exclusivity commitment would not bind an acquirer of us that owns or controls such a product so long as certain precautions are followed to ensure that CSL Behring’s confidential information and our proprietary technology related to the Product are not used or accessed by personnel of such acquirer who are developing or commercializing such competing product.
Unless earlier terminated as described below, the CSL Behring Agreement will continue on a country-by-country basis until expiration of the royalty term in a country. The royalty term expires in a country on the later of (a) 15 years after the first commercial sale of the Product in such country, (b) expiration of regulatory exclusivity for the Product in such country and (c) expiration of all valid claims of specific licensed patents covering the Product in such country. Either we or CSL Behring may terminate the CSL Behring Agreement for the other party’s material breach if such breach is not cured within a specified cure period. In addition, if CSL Behring fails to commercialize the Product in any of a group of major countries for an extended period of time following the first regulatory approval of the Product in any of such group of countries (other than due to certain specified reasons) and such failure has not been cured within a specified cure period, then we may terminate the CSL Behring Agreement. CSL Behring may also terminate the CSL Behring Agreement for convenience.

The effectiveness of the transactions contemplated by the CSL Behring Agreement is contingent on completion of review under antitrust laws in the United States, Australia, and the United Kingdom, and certain provisions of the CSL Behring Agreement will not become effective until after we receive all such regulatory approvals.

On November 11, 2020, the Australian Competition and Consumer Commission ("ACCC") determined, pursuant to section 50 of the Competition and Consumer Act 2010 of Australia, that it will not intervene in the CSL Behring Agreement. Thus, the ACCC has completed its review of the CSL Behring Agreement, and the transactions contemplated by the CSL Behring Agreement may close from the perspective of the Australian competition authority.

On November 24, 2020, the Competition and Markets Authority in the United Kingdom (the "CMA") adopted a decision not to refer the CSL Behring Agreement for proceedings under section 33 of the Enterprise Act 2002 of the United Kingdom. The decision was made public by the CMA on January 6, 2021. Thus, the CMA has completed its review of the CSL Behring Agreement, and the transactions contemplated by the CSL Behring Agreement may close from the perspective of the United Kingdom competition authority.

On December 3, 2020, we and CSL Behring filed a premerger notification and report form under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 (the "HSR Act"). On January 4, 2021, the United States Federal Trade Commission ("FTC") issued to us a request for additional information and documentary material (a "Second Request") under the HSR Act. The FTC similarly issued a Second Request to CSL Behring also with respect to the antitrust review of the CSL Behring Agreement. The effect of the Second Request is to extend the waiting period imposed under the HSR Act until 30 days after all parties to the CSL Behring Agreement have substantially complied with the requests unless the waiting period is terminated earlier by the FTC or voluntarily extended by the parties. We do not believe that the FTC will determine that the consummation of the transaction will result in a violation of the HSR Act. However, there can be no assurance as to the outcome of the Second Request.

Closing of the transaction is expected to materially impact our profitability and cash flows. Receipt of the $450.0 million payment due on closing would extend the funding of our operations into the second half of 2024 (assuming a full repayment of funds borrowed from Hercules Capital Inc. ("Hercules") under our term loan facility by 2023). However, we expect to continue to incur losses and to generate negative cash flows beyond the fiscal year in which we would close the transaction.

Hemophilia B program – Etranacogene dezaparvovec (AMT-061)

Etranacogene dezaparvovec is our lead gene therapy candidate and includes an Adeno-associated virus ("AAV") serotype 5 (together “AAV-5”) vector incorporating the functional human Factor IX ("FIX") Padua variant. We are currently conducting a pivotal study in patients with severe and moderately-severe hemophilia B.
In August 2018, we initiated a Phase Ib dose-confirmation study of etranacogene dezaparvovec and in September 2018, we completed the dosing for that study. In February, May, July, and December 2019, and in December 2020, we presented updated data from the Phase Ib dose-confirmation study of etranacogene dezaparvovec. The most recent data that we announced from the Phase Ib study of etranacogene dezaparvovec show that all three patients experienced increasing and sustained FIX levels after a one-time administration of etranacogene dezaparvovec. Mean FIX activity was 44.2% of normal two years after administration, exceeding threshold FIX levels generally considered sufficient to significantly reduce the risk of bleeding events. The first patient achieved FIX activity of 44.7% of normal, the second patient was 51.6% of normal and the third patient was 36.3% of normal. The second and third patients had previously screen-failed and were excluded from another gene therapy study due to pre-existing neutralizing antibodies to a different AAV vector. At two years after dosing, two of the three participants remain free from bleeds and use of FIX replacement therapy. A single bleed has been reported in one participant, who has used a total of two FIX infusions (excluding surgery). All patients have remained free of prophylaxis in the two years since receiving etranacogene dezaparvovec.

In June 2018, we initiated the six-month lead-in period of our Phase III HOPE-B pivotal trial of etranacogene dezaparvovec (the “HOPE-B trial”). The HOPE-B trial is a multinational, multi-center, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec. After the six-month lead-in period, patients received a single intravenous administration of etranacogene dezaparvovec. Patients enrolled in the HOPE-B trial were tested for the presence of pre-existing neutralizing antibodies to AAV5 but not excluded from the trial based on their titers.

The primary endpoints of the study are based on the FIX activity level achieved following the administration of etranacogene dezaparvovec after 26 weeks and 52 weeks after dosing as well as annualized bleed rates during the 52 weeks after dosing.

In March 2020, we completed dosing of the 54 patients in the HOPE-B trial. The targeted number of patients to be dosed per the clinical trial protocol was 50. As set forth below, we have implemented and continue to implement various measures to allow us to closely monitor the trial within the guidance provided by the U.S. Food and Drug Administration ("FDA") regarding the impact of the COVID-19 coronavirus pandemic ("COVID-19") to minimize any risk or disruption in patient follow-up visits.

In December 2020, we announced top-line data from the HOPE-B trial. The 26-week follow-up date from the trial showed that FIX activity in the 54 patients increased after dosing from ≤ 2% to a mean of 37.2% at 26 weeks, meeting a first primary endpoint of the HOPE-B trial. No correlation between pre-existing neutralizing antibodies and FIX activity was found in patients with neutralizing antibody titers up to 678.2, a range expected to include more than 95% of the general population; one patient with a neutralizing antibody titer of 3,212.3 did not show an increase in FIX activity. Less than 1% of the general population is expected to have neutralizing antibody titers of greater than 3,000.

During the 26-week period after dosing, 15 patients (28%) reported a total of 21 bleeding events, representing a reduction of 83% compared to the 123 bleeding events reported by 38 patients (70%) during the observational lead-in phase of the trial. Total bleeds include any bleeding event reported after the treatment of etranacogene dezaparvovec, including spontaneous, traumatic, and those associated with unrelated medical procedures, whether or not FIX treatment was required. Of the total bleeding events reported during the 26-week period after dosing, only three were classified as spontaneous bleeds requiring treatment, representing a reduction of 92% compared to the 37 such bleeding events reported during the observational lead-in phase. Mean annualized usage of FIX replacement therapy, a secondary endpoint in the clinical trial, declined by 96% during the 26-week period after dosing compared to the observational lead-in phase. Etranacogene dezaparvovec was generally well-tolerated. As of the November 2020 cut-off date, most adverse events were classified as mild (81.5%). The most common events included transaminase elevation treated with steroids per protocol (9 patients; 17%), infusion-related reactions (7 patients; 13%), headache (7 patients; 13%) and influenza-like symptoms (7 patients; 13%). Liver enzyme elevations resolved with a tapering course of corticosteroids and FIX activity remained in the mild range in the steroid treated patients. No relationship between safety and neutralizing antibody titers was observed. Based on interactions with the FDA and the European Medicines Agency ("EMA"), we plan to incorporate FIX activity and bleeding rates at 52 weeks as additional co-primary endpoints in the study.
On December 21, 2020, our clinical trials of etranacogene dezaparvovec, including our HOPE-B trial, were placed on clinical hold by the FDA. The clinical hold was initiated following the submission of a safety report in mid-December relating to a possibly related serious adverse event associated with a preliminary diagnosis of hepatocellular carcinoma (“HCC”), a form of liver cancer, in one patient in the HOPE-B trial that was treated with etranacogene dezaparvovec in October 2019. The patient has multiple risk factors associated with HCC, including a twenty-five-year history of hepatitis C (“HCV”), hepatitis B virus (“HBV”), evidence of non-alcoholic fatty liver disease and advanced age. Chronic infections with hepatitis B and C have been associated with approximately 80% of HCC cases.

The liver lesion was detected during a routine abdominal ultrasound conducted as part of the required study assessments in patients at one-year post dosing. A surgical resection of the lesion has occurred, and an analysis of the tissue samples was initiated in early 2021. On February 19, 2021, we reported initial results from this analysis to the FDA in accordance with pharmacovigilance requirements. We are gathering final data from these molecular analyses and will be preparing a detailed response to the FDA's clinical hold questions regarding this event. Currently, we do not have adequate data to determine a possible causal relationship, especially in the context of the other known risk factors. We currently do not anticipate any impact on our regulatory submission timelines, including the filing of a BLA.

No other cases of HCC have been reported in our clinical trials conducted in more than 67 patients in hemophilia B, with some patients dosed more than 5 years ago.

Etranacogene dezaparvovec has been granted Breakthrough Therapy Designation by the FDA and access to the current priority medicines (“PRIME”) initiative by the EMA.

Huntington’s disease program (AMT-130)

AMT-130 is our novel gene therapy candidate for the treatment of Huntington’s disease. AMT-130 utilizes our proprietary, gene-silencing miQURE platform and incorporates an AAV vector carrying an miRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment. AMT-130 has received orphan drug and Fast Track designations from the FDA and Orphan Medicinal Product Designation from the EMA.

In June 2020, we announced the completion of the first two patient procedures in the Phase I/II clinical trial of AMT-130 for the treatment of Huntington’s disease. These procedures occurred after a postponement that resulted from the COVID-19 pandemic and the associated states of emergency declarations in the United States. The Phase I/II protocol is a randomized, imitation surgery-controlled, double-blinded study conducted at three surgical sites, and multiple referring, non-surgical sites in the U.S. The primary objective of the study is to evaluate the safety, tolerability, and efficacy of AMT-130 at two doses.

On September 25, 2020, we announced that the independent Data Safety Monitoring Board (“DSMB”) overseeing the Phase I/II clinical trial of AMT-130 for the treatment of Huntington’s disease had met and reviewed 90-day safety data from the first two patients enrolled in the trial. No significant safety concerns were noted to prevent further dosing.

On October 13, 2020, we announced the completion of the third and fourth patient procedures in the Phase I/II clinical trial.

On February 8, 2021, we announced that the DSMB had met and reviewed the six-month safety data from the first two enrolled patients and the 90-day safety data from the next two enrolled patients in the study. No significant safety concerns were noted to prevent further dosing, and the final six patients in the first cohort are now cleared for enrollment.

BMS collaboration

We and Bristol-Myers Squibb (“BMS”) entered into a collaboration and license agreement in May 2015 (“BMS CLA”). BMS had initially designated four Collaboration Targets in 2015 and in accordance with the terms of the BMS CLA could have designated a fifth to tenth Collaboration Target.

In February 2019, BMS requested a one-year extension of the initial research term. In April 2019, following an assessment of the progress of this collaboration and our expanding proprietary programs, we notified BMS that we did not intend to agree to an extension of the initial research term. Accordingly, the initial four-year research term under the collaboration terminated on May 21, 2019.

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