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): **June 20, 2023**

uniQure N.V.
(Exact Name of Registrant as Specified in Charter)

The Netherlands	**001-36294**	**N/A**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

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

Securities registered pursuant to Section 12(b) of the Act:

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 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 ☐

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. ☐

Item 7.01 Regulation FD Disclosure.

On June 20, 2023, uniQure N.V. (the "Company") issued a press release announcing achievement of a $100 million milestone associated with the first commercial sale of HEMGENIX® (etranacogene dezaparvovec) in the United States by its partner, global biotechnology leader CSL Behring. A copy of the press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and incorporated herein by reference.

On June 21, 2023, the Company issued a press release announcing updates on its ongoing U.S. Phase I/II clinical trial of AMT-130 gene therapy for the treatment of Huntington's disease, as described in more detail in Item 8.01 below. A copy of the press release is attached to this Current Report on Form 8-K as Exhibit 99.2 and incorporated herein by reference.

The information provided in this Item 7.01, including the accompanying Exhibits 99.1 and 99.2, shall be deemed "furnished" and shall not be deemed "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of such section, nor shall it be incorporated by reference in any filing made by the Company pursuant to the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, regardless of the general incorporation language of such filing, except to the extent that such filing incorporates by reference any or all of such information by express reference.

Item 8.01 Other Events.

On June 21, 2023, the Company announced promising interim data relating to its ongoing U.S. Phase I/II clinical trial of AMT-130, a one-time administered investigational gene therapy for Huntington's disease. The interim data includes up to 24 months of follow-up data from the 26 patients enrolled in the ongoing U.S. clinical trial. The trial includes a 10-patient low-dose cohort (six treated patients and four control patients) and a 16-patient high-dose cohort (10 treated patients and six control patients). Patients were randomized to treatment with AMT-130 or an imitation (sham) surgery. The study consists of a blinded 12-month core study period followed by unblinded long-term follow-up of five years for treated patients. To date, four of the six control patients in the high-dose cohort have been crossed over to treatment. Efficacy and biomarker data from the crossover patients were not included in the interim data update.

Safety and Tolerability

AMT-130 was generally well-tolerated with a manageable safety profile in patients treated with the lower dose of $6x10^{12}$ vector genomes and the higher dose of $6x10^{13}$ vector genomes. The most common adverse events in the treatment groups were related to the surgical procedure. No treatment emergent adverse events led to discontinuation of patient follow-up.

As previously reported, there were two serious adverse events (SAE) unrelated to AMT-130 (post-operative delirium and major depression) in the low-dose cohort, one SAE in the high-dose cohort (back pain), and one SAE (deep vein thrombosis) in the control group. In addition, there were two suspected unexpected SAEs (severe headache, central nervous system inflammation) in the high-dose cohort. All the events have resolved.

All four crossover patients (three high dose, one low dose) received a short course of immunosuppression therapy concurrent with the administration of AMT-130. Following a review of the interim data analysis, the Data Safety Monitoring Board for the U.S. Phase I/II clinical trial concluded there are no safety concerns with either dose and recommended continuing clinical development of AMT-130.

Exploratory Efficacy Data

Clinical and functional measurements for treated patients in each dose cohort were compared to baseline measurements, control patients (up to 12 months) and a natural history cohort. The natural history cohort was developed by the Company in collaboration with the Cure Huntington's Disease Initiative using the TRACK-HD natural history study of patients with early Huntington's disease. The natural history cohort includes 31 patients that met the Company's clinical trial inclusion-criteria of CAG length, age, Total Functional Capacity, Diagnostic Classification Level and minimum striatal volumes.

- Early clinical data demonstrate trends consistent with a potential clinical benefit of AMT-130 at both doses of AMT-130.

- Compared to baseline measurements, clinical function was generally preserved at 24 months for patients in the low-dose cohort and at 12-months for patients in the high-dose cohort.

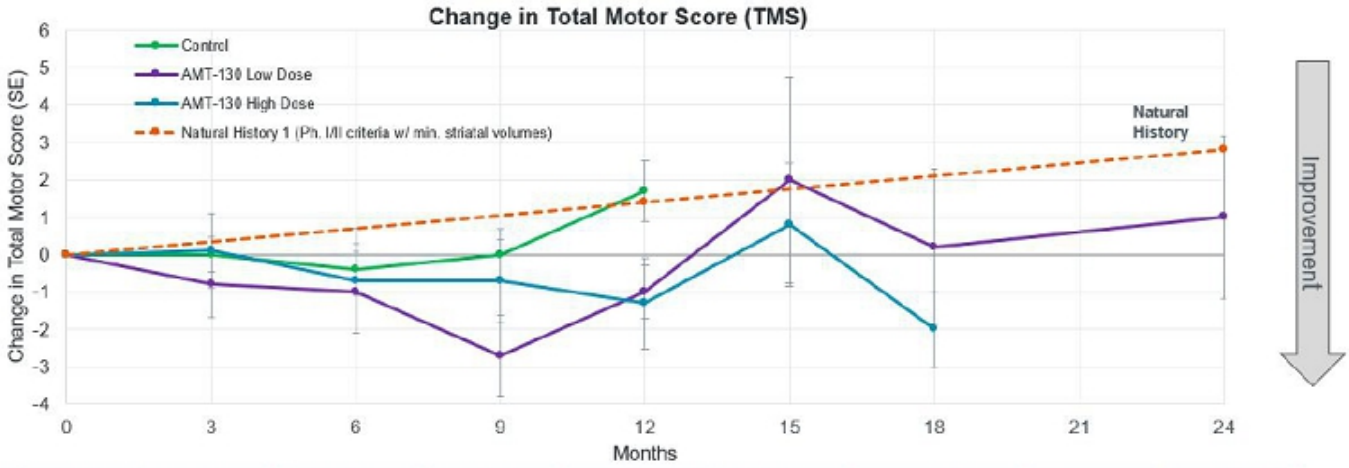
- Compared to the natural history, patients in both dose cohorts demonstrated benefits in each of Total Motor Score, Total Functional Capacity and the composite Unified Huntington's Disease Rating Scale.

    - *Total Motor Score (TMS)*: Low-dose patients demonstrated a mean improvement in TMS of 1.8 points at 24 months compared to the natural history and high-dose patients demonstrated a mean improvement of 2.7 points at 12 months.

    - *Total Functional Capacity (TFC)*: Low-dose patients demonstrated a mean 0.8 point improvement in TFC at 24 months compared to the natural history and high-dose patients demonstrated a mean 0.5 point improvement at 12 months.

    - *Composite Unified Huntington's Disease Rating Scale (cUHDRS)*: Low-dose patients demonstrated a mean 0.9 point improvement in cUHDRS at 24 months compared to the natural history and high-dose patients demonstrated a mean 1.0 point improvement at 12 months.

- Patients in the control group experienced a worsening of TMS at 12 months compared to baseline measurements and the natural history. TFC and cUHDRS was preserved in control patients at 12 months.



Patients	Base	3M	6M	9M	12M	15M	18M		24M
Low Dose	6	6	6	6	6	6	6		5
High Dose	10	10	10	10	9	4	2		
Control	10	10	10	10	9				

Biomarkers

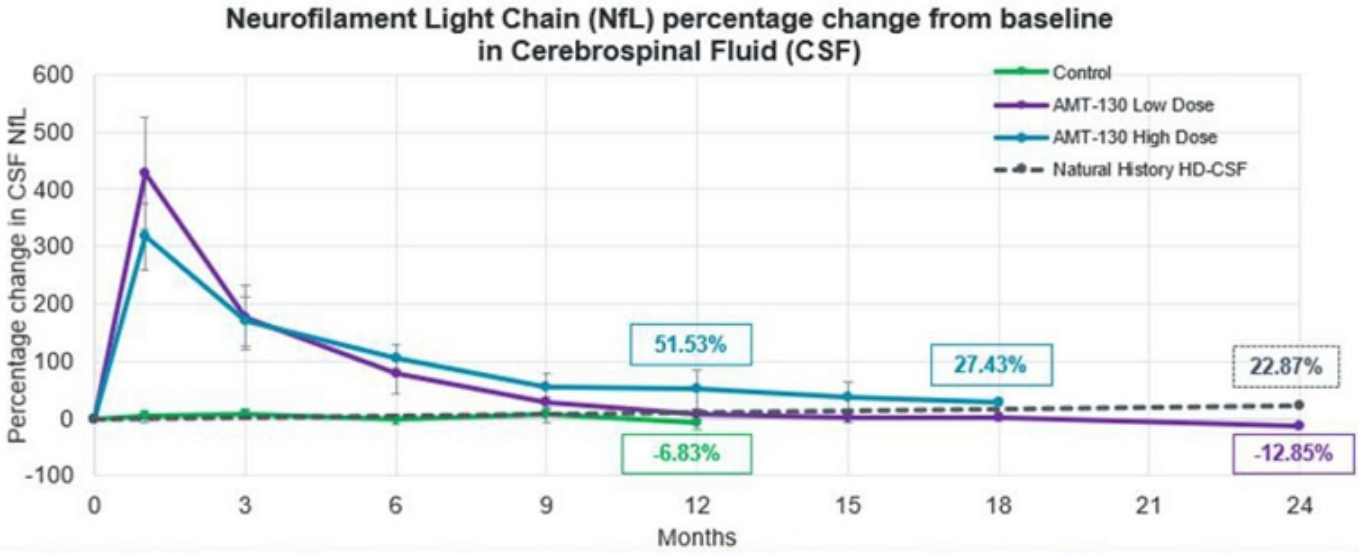
Neurofilament light chain (NfL)

- As expected and as previously reported, patients treated with AMT-130 experienced a transient increase in cerebrospinal fluid (CSF) NfL related to the procedure that peaked at approximately one month after administration. These transient increases were not dose-dependent and all patients experienced subsequent declines in CSF NfL.

- Mean CSF NfL for the low-dose cohort was 12.9% below baseline measurements compared to a predicted 22.9% increase in the natural history, with four of the five low-dose patients having CSF NfL levels below baseline measurements.

- CSF NfL levels in the high-dose cohort were more variable through 12 months, with a mean increase of 51.5% compared to baseline. Four of the eight high-dose patients with at least 12 months of follow up had NfL levels below baseline measurements. Two high-dose patients with 18 months of follow-up demonstrated a continued decline in CSF NfL to 27.4% above baseline.

- In the control group, mean CSF NfL was relatively stable and was 6.83% below baseline measurements at 12-months.



Patients	Base	1M	3M	6M	9M	12M	15M	18M		24M
Low-Dose	6	6	5	6	6	6	6	6		5
High-Dose	10	10	9	10	10	8	4	2		
Control	10	9	9	9	7	7				

Mutant Huntingtin protein (mHTT)

- CSF mHTT for the low-dose cohort remained below baseline measurements with a mean reduction of 8.1% at 24 months. CSF mHTT for the high-dose cohort was significantly more variable with a mean increase of 39.7% above baseline at 12 months compared to a 4.7% increase in the control group. Three of nine evaluable patients in the high-dose cohort had CSF mHTT reduction below baseline measurements at their last measurement.

Total Brain Volume

- The mean total brain volume for the control, low-dose and high-dose cohorts declined 0.74%, 1.02% and 1.23%, respectively at 12 months and were not significantly different from each other or from the natural history.

Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as “anticipate,” “believe,” “could,” “establish,” “estimate,” “expect,” “goal,” “intend,” “look forward to,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “will,” “would” and similar expressions and the negatives of those terms. Forward-looking statements are based on management’s beliefs and assumptions and on information available to management only as of the date of this Current Report on Form 8-K. These forward-looking statements include, but are not limited to, the potential clinical and functional effects of AMT-130. The Company’s actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including risks related to conducting the clinical trial for Huntington’s disease, the impact of financial and geopolitical events on the Company and the wider economy and health care system, the Company’s clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading “Risk Factors” in the Company’s periodic securities filings, including its Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 27, 2023 and its Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 9, 2023. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
99.1	Press Release of uniQure N.V. dated June 20, 2023 announcing achievement of a $100 million milestone related to hemophilia B gene therapy (furnished solely for purposes of Item 7.01 of this Current Report on Form 8-K).
99.2	Press Release of uniQure N.V. dated June 21, 2023 announcing update on U.S. Phase I/II clinical trial of AMT-130 gene therapy for the treatment of Huntington's disease (furnished solely for purposes of Item 7.01 of this Current Report on Form 8-K).
104	Cover Page Interactive Data File (embedded with the Inline XBRL document).

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 22, 2023

By: /s/ Jeannette Potts
JEANNETTE POTTS
Chief Legal and Compliance Officer

Exhibit 99.1

uniQure announces achievement of $100 million milestone related to hemophilia B gene therapy

~ Milestone payment triggered by first commercial sale of HEMGENIX® in U.S. by CSL Behring ~

Lexington, MA and Amsterdam, the Netherlands, June 20, 2023 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, announced the achievement of a $100 million milestone associated with the first commercial sale of HEMGENIX® in the United States by its partner, global biotechnology leader CSL (ASX: CSL). HEMGENIX® (etranacogene dezaparvovec) is a one-time administered gene therapy for the treatment of adults with hemophilia B who currently use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage or have repeated, serious spontaneous bleeding episodes.

"The first commercial sale in the U.S. is a major milestone for uniQure as it marks the fulfillment of our promise to deliver genetic medicines that have the potential to transform people's lives," said Matt Kapusta, chief executive officer of uniQure. "uniQure's successful development of HEMGENIX® further validates our AAV platform built on the back of 25 years of scientific leadership and innovation in the field of gene therapy. We look forward to our continued collaboration with CSL Behring as they work to bring this important treatment to those living with hemophilia B."

uniQure conducted the multi-year research and clinical development program for HEMGENIX®, which included three clinical trials across 34 global sites and involved 67 adults with hemophilia B. In May 2021, uniQure and CSL completed a licensing transaction providing CSL Behring with exclusive rights to commercialize and continue clinical development of HEMGENIX® globally. uniQure is responsible for the global manufacturing of the product at its licensed facility in Lexington, Massachusetts. Under the agreement with CSL Behring, the milestone payment is due within 30 days after achievement of the milestone.

HEMGENIX® is the first approved gene therapy for hemophilia B in the United States, European Union (EU) and European Economic Area (EEA), and the UK.

Hemophilia B is a rare, lifelong bleeding disorder caused by a single gene defect, resulting in insufficient production of factor IX, a protein primarily produced by the liver that helps blood clots form. Treatments for moderate to severe hemophilia B include prophylactic infusions of factor IX replacement therapy to temporarily replace or supplement low levels of blood-clotting factor and, while these therapies are effective, those with hemophilia B must adhere to strict, lifelong infusion schedules. They may also still experience spontaneous bleeding episodes as well as limited mobility, joint damage or severe pain as a result of the disease. For appropriate patients, HEMGENIX® has been shown in clinical trials to allow people living with hemophilia B to produce their own factor IX, which can lower the risk of bleeding.

About HEMGENIX

HEMGENIX® is a gene therapy that reduces the rate of abnormal bleeding in eligible people with hemophilia B by enabling the body to continuously produce factor IX, the deficient protein in hemophilia B. It uses AAV5, a non-infectious viral vector, called an adeno-associated virus (AAV). The AAV5 vector carries the Padua gene variant of Factor IX (FIX-Padua) to the target cells in the liver, generating factor IX proteins that are 5x-8x more active than normal. These genetic instructions remain in the target cells, but generally do not become a part of a person's own DNA. Once delivered, the new genetic instructions allow the cellular machinery to produce stable levels of factor IX.

Important Safety Information (ISI)

What is HEMGENIX?

HEMGENIX®, etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

Please see full prescribing information for HEMGENIX.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The recent approval of our gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represents a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. We are now leveraging our modular and validated technology platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, ALS, Fabry disease, and other severe 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," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "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 about whether we are able to bring AMT-061 to people living with hemophilia B and whether the treatment will be transformational. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the postponement in our clinical trial for Huntington's disease, the impact of financial and geopolitical events on our Company and the wider economy and health care system, our Commercialization and License Agreement with CSL Behring, our clinical development activities, clinical results, collaboration arrangements, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in the Company's periodic securities filings, including its Annual Report on Form 10-K filed February 28, 2023. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and the Company assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

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Exhibit 99.2

uniQure Announces Update on U.S. Phase I/II Clinical Trial of AMT-130 Gene Therapy for the Treatment of Huntington's Disease

~ AMT-130 continues to be generally well-tolerated across both dose cohorts ~

~ Patients treated with AMT-130 show preserved function compared to baseline and clinical benefits relative to natural history of the disease ~

~ Neurofilament Light Chain (NfL) in cerebrospinal fluid (CSF) was below baseline at 24 months in patients treated with the low-dose of AMT-130 and declining towards baseline at 12 months in patients treated with the high-dose of AMT-130 ~

~ Suppression of CSF mHTT in low-dose cohort supports AMT-130 target engagement; Greater variability observed in high-dose cohort ~

~ Promising data support continuing clinical development of AMT-130 and pursuing regulatory interactions to discuss late-stage development ~

~ Investor conference call and webcast today at 8:30 a.m. ET ~

Lexington, MA and Amsterdam, the Netherlands, June 21, 2023 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced promising interim data, including up to 24 months of follow-up, from 26 patients enrolled in the ongoing U.S. Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease.

"We are very pleased with the data from the interim analysis of our U.S. Phase I/II clinical trial of AMT-130, a one-time administered investigational gene therapy for Huntington's Disease." stated Ricardo Dolmetsch, Ph.D., president of research and development at uniQure. "AMT-130 continues to be generally well-tolerated with a manageable safety profile at both doses. Importantly, both doses show preliminary evidence of clinical and functional benefits, including favorable trends in Total Motor Score, Total Functional Capacity and the composite Unified Huntington's Disease Rating Scale compared to natural history. We plan to engage with regulators to advance this promising clinical program as we collect more data from these patients and from our European study."

"Today's encouraging interim update shows early signs of a potential clinical benefit of AMT-130 and supportive trends in neurofilament light chain, a key marker of neuronal damage that has proven useful across multiple neurodegenerative disorders," stated Sarah Tabrizi, M.D., FRCP, Ph.D., professor of clinical neurology, director of the University College London (UCL) Huntington's Disease Center and joint head of the department of neurodegenerative disease at UCL. "Despite the small patient numbers, I am encouraged to see that patients treated with either dose of AMT-130 appear to have largely preserved function and are trending favorably to natural disease course at up to 24 months. These interim results provide early hope for patients suffering from this devastating disease, and I look forward to additional clinical updates and the further investigation of AMT-130 as a potentially important treatment option for patients with Huntington's disease."

Data Summary from the U.S. Phase I/II Trial of AMT-130 in Huntington's Disease

A total of 26 patients with early-manifest Huntington's disease have been enrolled in the multi-center, U.S. Phase I/II clinical trial of AMT-130, including a 10-patient low-dose cohort (6 treated, 4 control) and a 16-patient, high-dose cohort (10 treated, 6 control). Patients were randomized to treatment with AMT-130 or an imitation (sham) surgery. The study consists of a blinded 12-month core study period followed by unblinded long-term follow-up of five years for treated patients. To date, four of the six control patients in the high dose cohort have been crossed over to treatment. Efficacy and biomarker data from the crossover patients are not included in the summary below.

Safety and Tolerability

AMT-130 was generally well-tolerated, with a manageable safety profile in patients treated with the lower dose of $6x10^{12}$ vector genomes and the higher dose of $6x10^{13}$ vector genomes. The most common adverse events in the treatment groups were related to the surgical procedure. No treatment emergent adverse events (TEAEs) led to discontinuation of patient follow-up.

As previously reported, there were two serious adverse events (SAE) unrelated to AMT-130 (post-operative delirium and major depression) in the low-dose cohort, one SAE in the high-dose cohort (back pain), and one SAE (deep vein thrombosis) in the control group. In addition there were two suspected unexpected serious adverse events (severe headache, central nervous system inflammation) in the high-dose cohort. All the events have resolved.

All four crossover patients (3 high dose, 1 low dose) received a short course of immunosuppression therapy concurrent with the administration of AMT-130. Following a review of the interim data analysis, the Data Safety Monitoring Board (DSMB) for the U.S. Phase I/II clinical trial concluded there are no safety concerns with either dose and recommended continuing clinical development of AMT-130.

Exploratory Efficacy Data

Clinical and functional measurements for treated patients in each dose cohort were compared to baseline measurements, control patients (up to 12 months) and a natural history cohort. The natural history cohort was developed by uniQure in collaboration with the Cure Huntington's Disease Initiative (CHDI) using the TRACK-HD natural history study of patients with early Huntington's disease. The cohort includes 31 patients that met the uniQure clinical trial inclusion-criteria of CAG length, age, Total Functional Capacity, Diagnostic Classification Level and minimum striatal volumes.

- Early clinical data demonstrate trends consistent with a potential clinical benefit of AMT-130 at both doses of AMT-130.
- Compared to baseline measurements, clinical function was generally preserved at 24 months for patients in the low-dose cohort and at 12 months for patients in the high-dose cohort.
- Compared to natural history, patients in both dose cohorts demonstrated benefits in each of Total Motor Score, Total Functional Capacity and the composite Unified Huntington's Disease Rating Scale.
    - *Total Motor Score (TMS):* Low-dose patients demonstrated a mean improvement in TMS of 1.8 points at 24 months compared to natural history and high-dose patients demonstrated a mean improvement of 2.7 points at 12 months.
    - *Total Functional Capacity (TFC)*: Low-dose patients demonstrated a mean 0.8 point improvement in TFC at 24 months compared to natural history and high-dose patients demonstrated a mean 0.5 point improvement at 12 months.

- *Composite Unified Huntington's Disease Rating Scale (cUHDRS)*: Low-dose patients demonstrated a mean 0.9 point improvement in cUHDRS at 24 months compared to natural history and high-dose patients demonstrated a mean 1.0 point improvement at 12 months.

- Patients in the control group experienced a worsening of Total Motor Score at 12 months compared to baseline and natural history. TFC and cUHDRS was preserved in control patients at 12 months.

Patients	Base	3M	6M	9M	12M	15M	18M		24M
Low Dose	6	6	6	6	6	6	6		5
High Dose	10	10	10	10	9	4	2		
Control	10	10	10	10	9				

Biomarkers

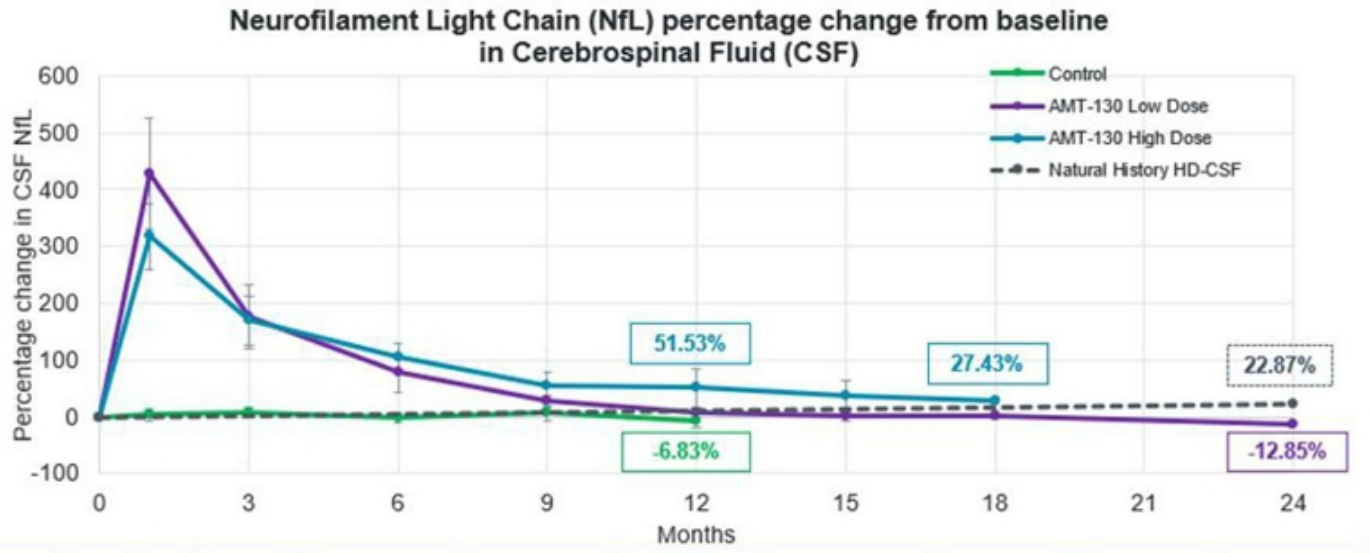
Neurofilament light chain (NfL)

- As expected and as previously reported, patients treated with AMT-130 experienced a transient increase in CSF NfL related to the procedure that peaked at approximately one month after administration. These transient increases were not dose-dependent and all patients experienced subsequent declines in CSF NfL.

- Mean CSF NfL for the low-dose cohort was 12.9% below baseline compared to a predicted 22.9% increase in the natural history, with four of the five low-dose patients having CSF NfL levels below baseline.

- CSF NfL levels in the high-dose cohort were more variable through 12 months, with a mean increase of 51.5% compared to baseline. Four of the eight high-dose patients with at least 12 months of follow-up had NfL levels below baseline. Two high-dose patients with 18 months of follow-up demonstrated a continued decline in CSF NfL to 27.4% above baseline.

- In the control group, mean CSF NfL was relatively stable and was 6.83% below baseline at 12 months.



Patients	Base	1M	3M	6M	9M	12M	15M	18M		24M
Low-Dose	6	6	5	6	6	6	6	6		5
High-Dose	10	10	9	10	10	8	4	2		
Control	10	9	9	9	7	7				

Mutant Huntingtin protein (mHTT)

- CSF mHTT for the low-dose cohort remained below baseline with a mean reduction of 8.1% at 24 months. CSF mHTT for the high-dose cohort was significantly more variable with a mean increase of 39.7% above baseline at 12 months compared to a 4.7% increase in the control group. Three of nine evaluable patients in the high-dose cohort had CSF mHTT reduction below baseline at their last measurement.

Total Brain Volume

- The mean total brain volume for the control, low-dose and high-dose cohorts declined 0.74%, 1.02% and 1.23%, respectively at 12 months and were not significantly different from each other or from the natural history.

Next Steps

Based on the promising data from this interim analysis, uniQure will advance the clinical development of AMT-130 and anticipates the following next steps:

- Early in the third quarter of 2023, uniQure expects to complete patient enrollment in the high-dose cohort of the European clinical trial.
- In the second half of 2023, uniQure expects to initiate a third cohort in the ongoing U.S. clinical trial to further investigate both doses in combination with perioperative immunosuppression with a focus on evaluating near-term safety. The third cohort will enroll up to 10 patients, all of whom will receive AMT-130 using the current, established stereotactic neurosurgical delivery procedure.
- In the fourth quarter of 2023, uniQure expects to present new clinical data from the Phase I/II studies of AMT-130, including additional follow-up data from the treated patients in the U.S. trial and 12-month follow-up data from the low-dose patients in the EU trial.

- By the first quarter of 2024, uniQure anticipates holding regulatory interactions to discuss data from the U.S. and EU studies and the pathway for further advancing the clinical development of AMT-130.

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Wednesday, June 21, 2023 at

8:30 a.m. ET. The event will be webcast under the Events & Presentations section of uniQure's website at https://www.uniqure.com/investors-media/events-presentations and following the event a replay will be archived for 90 days. Interested parties participating by phone will need to register using this online form. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone. If you are joining the conference call, please dial in 15 minutes before the start time.

About the Phase I/II Clinical Program of AMT-130

The U.S. Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease is exploring the safety, tolerability, and efficacy signals in 26 total patients with early manifest Huntington's disease split into a 10 patient, low-dose cohort followed by a 16 patient, high-dose cohort; patients are randomized to treatment with AMT-130 or an imitation (sham) surgery. The multi-center trial consists of a blinded 12-month core study period followed by unblinded long-term follow-up for five years. A total of 16 patients in the clinical trial were randomized to treatment and received a single administration of AMT-130 through MRI-guided, convection-enhanced stereotactic neurosurgical delivery directly into the striatum (caudate and putamen). An additional four control patients in the high-dose cohort crossed over to treatment. Additional details are available on www.clinicaltrials.gov (NCT04120493).

The European, open-label Phase Ib/II study of AMT-130 will enroll 15 patients with early manifest Huntington's disease across two dose cohorts. The low-dose cohort of six patients has completed enrollment with the remaining high-dose cohort expected to complete enrollment in mid-2023. Together with the U.S. study, the European study is intended to establish safety, proof of concept, and the optimal dose of AMT-130 to take forward into Phase III development or into a confirmatory study should an accelerated registration pathway be feasible.

AMT-130 is uniQure's first clinical program focusing on the central nervous system (CNS) incorporating its proprietary miQURE® platform.

About Huntington's Disease

Huntington's disease is a rare, inherited neurodegenerative disorder that leads to motor symptoms including chorea, and behavioral abnormalities and cognitive decline resulting in progressive physical and mental deterioration. The disease is an autosomal dominant condition with a disease-causing CAG repeat expansion in the first exon of the huntingtin gene that leads to the production and aggregation of abnormal protein in the brain. Despite the clear etiology of Huntington's disease, there are no currently approved therapies to delay the onset or to slow the disease's progression.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The recent approvals of uniQure's gene therapy for hemophilia B – a historic achievement based on more than a decade of research and clinical development – represents a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. uniQure is now leveraging its modular and validated technology platform to advance a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, ALS, Fabry disease, and other severe diseases. www.uniQure.com

uniQure Forward-Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "establish," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "seek," "should," "will," "would" and similar expressions and the negatives of those terms. 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, the potential clinical and functional effects of AMT-130, including as an important treatment option for patients with Huntington's disease, the expected completion of enrollment of the European, open-label Phase Ib/II study of AMT-130, and the initiation of a third cohort in the ongoing U.S. Phase I/II clinical. uniQure's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including risks related to conducting the clinical trial for Huntington's disease, the impact of financial and geopolitical events on uniQure and the wider economy and health care system, uniQure's clinical development activities, clinical results, collaboration arrangements, 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 periodic securities filings, including its Annual Report on Form 10-K filed with the SEC on February 27, 2023 and its Quarterly Report on Form 10-Q filed with the SEC on May 9, 2023. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and uniQure assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

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