# 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): **November 19, 2018**

# 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):

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o 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 8.01. Other Events**

Press Release of uniQure N.V. dated November 19, 2018 entitled “uniQure Highlights Pipeline Expansion and Advancements in Technology at Research & Development Day”.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits

99.1 Press Release of uniQure N.V. dated November 19, 2018 entitled “uniQure Highlights Pipeline Expansion and Advancements in Technology at Research & Development Day”.

**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: November 19, 2018

By: /S/ MATTHEW KAPUSTA
Matthew Kapusta
Chief Executive Officer

**Exhibit 99.1**

**uniQure Highlights Pipeline Expansion and Advancements in Technology at Research & Development Day**

*~ Unveils New AAV Gene Therapy Approaches to Hemophilia A, Fabry Disease and Spinocerebellar Ataxia Type 3 ~*

*~ Introduces miQURE™ Gene Silencing Platform with Applications Across Multiple Indications ~*

*~ Highlights Advancements in Manufacturing and Research Technology ~*

**Lexington, MA and Amsterdam, the Netherlands,** November 19, 2018 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced the expansion of its research pipeline with novel AAV gene therapy approaches to treating Hemophilia A, Fabry disease and Spinocerebellar Ataxia Type 3 at the Company's Research & Development Day held this morning in New York City.

"We are very proud of the progress the Company has made to deliver extensive preclinical data for these new gene therapy programs that expand our pipeline and further validate uniQure's potential best-in-class vector delivery platform," stated Sander van Deventer, M.D., Ph.D., chief scientific officer at uniQure. "The addition of these gene therapy candidates for indications in the liver and CNS brings us yet another step closer towards uniQure's goal of delivering transformational medicine to patients suffering from genetic diseases. We look forward to advancing these programs closer to the clinic in 2019."

**New Gene Therapy Programs**

- *Introduced new product candidate AMT-180, a novel hemophilia A gene therapy that has the potential to treat all hemophilia A patients including those with past and current inhibitors.*
    - Approximately 30 percent of patients with severe hemophilia A will develop an inhibitor that neutralizes the infused Factor VIII (FVIII) activity. This patient population has in the past been excluded from gene therapy approaches in clinical development.
    - AMT-180 is a one-time, intravenously-administered, AAV5-based gene therapy incorporating a proprietary modified Factor IX gene, Super9™, that has been demonstrated in preclinical studies to circumvent inhibitors to FVIII.
    - A proof-of-concept study demonstrated that administration of Super9 resulted in clinically relevant FVIII mimetic activity in hemophilia A mice and was not associated with hypercoagulability in wild-type mice.
    - Another study in non-human primates demonstrated that a single dose of AMT-180 resulted in expression levels that translate into FVIII mimetic activity expected to be clinically relevant in hemophilia A patients with or without inhibitors. In addition, Super9 induced clinically relevant thrombin activation in FVIII-depleted human plasma with or without inhibitors.
    - These data show that AMT-180 may lead to durable expression in hemophilia A patients and may provide long-term prevention of bleeds.
- *Introduced new product candidate AMT-190, a differentiated gene therapy for the treatment of Fabry disease.*

- Fabry disease is an inherited lysosomal storage disorder caused by a defect in a gene that encodes for a protein called α-galactosidase A (GLA). The GLA protein is an essential enzyme required to breakdown globotriaosylsphingosine (Gb3) and lyso-globotriaosylsphingosine (lyso-Gb3). In patients living with Fabry disease, Gb3 and lyso-Gb3 accumulate in various cells throughout the body causing progressive clinical signs and symptoms of the disease. Current treatment options, which consist of bi-weekly intravenous enzyme replacement therapy, have no therapeutic benefit in patients with advanced renal or cardiac disease. Studies have also shown that a majority of male patients develop antibodies that inhibit the GLA protein and interfere with therapeutic efficacy.
- AMT-190 is a one-time, intravenously-administered, AAV5-based gene therapy designed to circumvent GLA antibodies that can inhibit efficacy in Fabry patients. AMT-190 incorporates a modified version of α-N-acetylgalactosaminidase (NAGA), a protein that is structurally similar to the GLA protein but is not recognized by GLA-neutralizing antibodies. As such, AMT-190 has the potential to be a more effective, longer-term treatment of Fabry disease.
- In cultured cells and in a study in wild-type mice, AMT-190 resulted in clinically relevant GLA activity.
- In a preclinical proof-of-concept study, Fabry mice were injected with a single dose of AMT-190, resulting in modified NAGA expression with subsequent GLA-activity in plasma. At 2 and 4 weeks post-dosing, this GLA activity already translated to up to 50 percent reduction in lyso-Gb3 levels.
- These studies demonstrate proof-of-concept of AMT-190 as a gene therapy candidate for Fabry disease. A one-time administration of AMT-190 could potentially lead to long-term expression of GLA in the liver, kidneys and heart, with no loss of expression due to inhibitors.

- *Introduced new gene therapy candidate AMT-150, a novel treatment for Spinocerebellar Ataxia Type 3, a central nervous system disorder.*

  - Spinocerebellar Ataxia Type 3 (SCA3), also known as Machado-Joseph disease, is caused by a CAG-repeat expansion in the ATXN3 gene that results in an abnormal form of the protein ataxin-3. People with SCA3 experience brain degeneration that results in movement disorders, rigidity, muscular atrophy and paralysis. There is currently no treatment available that slows the progressive course of this lethal disease.
  - AMT-150 is a one-time, intrathecally-administered, AAV gene therapy incorporating the Company's proprietary miQURE™ silencing technology that is designed to halt ataxia in early manifest SCA3 patients.
  - In an *in-vitro* study with human Induced Pluripotent Stem (IPS) derived neurons, AMT-150 has been shown to lower the human ataxin-3 protein by 65 percent, without any off-target effects. The Company also performed a proof-of-concept in-life study in SCA3 mice demonstrating that AMT-150 was able to lower toxic ataxin-3 protein by 65 percent in the brain stem after a single administration. Further studies in non-human primates demonstrate the ability to distribute and express a reporter gene at a clinically relevant level in the most degenerated brain regions in SCA3.
  - These preclinical studies show that a single administration of AMT-150 results in sustained expression and efficient processing with on-target engagement. They also show that AMT-150 appears to be safe due to the lack of off-target activity. The Company is currently performing studies in large animals to demonstrate further safety and efficacy.

**Advances in Technology and Manufacturing**

- *Presented the Company's miQURE™ technology — a proprietary, next-generation gene silencing platform.*

    - miQURE is uniQure's novel technology platform designed to degrade disease-causing genes, without off-target toxicity, and induce silencing of the entire target organ through secondary exosome-mediated delivery. Gene therapy candidates designed with miQURE incorporate proprietary, therapeutic miRNA constructs that can be delivered using AAVs to potentially provide long-lasting activity.
    - Preclinical studies of miQURE-based gene therapies have demonstrated several important advantages, including enhanced tissue-specificity, improved nuclear and cytoplasmic gene lowering and no off-target effects associated with impact to the cellular miRNA or mRNA transcriptome. miQURE technology has been incorporated in AMT-130, an investigational gene therapy for Huntington's disease, and is expected to be applied to AMT-150 for SCA3.

- *Announced Advances in Manufacturing and Research Technology.*

    - uniQure presented data on a highly potent, next-generation promoter for liver-directed gene therapies. Two preclinical studies demonstrate that the optimized liver promoter can generate up to 40 times more protein expression compared to the reference promoter. The optimized promoter will be incorporated in the Company's gene therapy candidate AMT-180 for the treatment of hemophilia A.
    - The Company highlighted the advantages of uniQure's patent-protected Dual Baculovirus manufacturing technology, including reduced variability, higher vector purity and easier scalability.

A replay of the webcast from the Company's Research and Development Day will be available on the Investor section of the corporate website at www.uniQure.com along with a copy of the presentation .

**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, the achievement of any of our planned near term or other milestones, the development of our gene therapy product candidates including each of the product candidates at the pre-clinical stage of development, the ability to achieve therapeutic effects in human patients in any of our product candidates, the ability to produce a product candidate that is safe and effective, the ability to obtain regulatory approval for any of our product candidates, 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 collaboration activities, product*

*development activities, 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 and Quarterly Report on Form 10-Q filed on November 6, 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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