uniQure Announces Second Quarter 2026 Financial Results and Provides Company Update

July 29, 2026

~ U.S. and U.K. regulatory submissions for AMT-130 for Huntington's disease on track as planned for the third quarter of 2026 ~

~ Topline four-year data from the Phase I/II study of AMT-130 expected in September 2026 ~

~ Reported data from the first cohort in the Phase I/IIa trial of AMT-260 for refractory mesial temporal lobe epilepsy showed early biological signals of therapeutic activity and a favorable safety profile ~

~ Strengthened financial position with $259 million follow-on offering, extending cash runway into 2030 and funding the anticipated commercial launch of AMT-130 and continued pipeline investment ~

~ uniQure to host earnings call at 8:30 a.m. ET ~

LEXINGTON, Mass. and AMSTERDAM, July 29, 2026 (GLOBE NEWSWIRE) -- 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 the second quarter of 2026 and highlighted recent progress across its business.

"This has been a defining quarter – not just for uniQure, but for the Huntington's disease community," said Matthew Kapusta, chief executive officer at uniQure. "Following a productive Type B meeting with the FDA, we remain on track to submit our BLA for AMT-130 in the third quarter — a milestone that reflects years of rigorous science, disciplined execution, and an unwavering commitment to the patients and families living with this devastating disease. With four-year data expected in September and the U.K. regulatory activities progressing as planned, we enter the second half of 2026 with real momentum and a clear line of sight to potentially bringing this therapy to the people who need it."

"Beyond AMT-130, we continue to execute across our broader pipeline," Mr. Kapusta continued. "With a strong balance sheet, we believe we are well-positioned for what will be a transformative period for uniQure and the patients we serve."

Recent Company Developments and Updates

Advancing AMT-130 for the treatment of Huntington's disease

- In June 2026, the Company held a Type B meeting with the U.S. Food and Drug Administration (FDA). Official meeting minutes received in July 2026 confirmed that the FDA and the Company reached alignment that a Biologics License Application (BLA) submission under the accelerated approval pathway for AMT-130, based on the existing clinical data, is reasonable. In addition, the FDA seeks to align on the confirmatory study design prior to the BLA submission, including consideration of a randomized standard-of-care control design instead of a sham procedure. The FDA also stated that, in accordance with the FDA's draft public guidance for accelerated approvals, the confirmatory study should be feasible to conduct within a reasonable timeline and be well underway, and potentially fully enrolled, at the time of accelerated approval.
- Discussions with the FDA to align on the confirmatory study design and analysis are underway and the Company expects to submit a BLA in the third quarter of 2026. The Company plans to initiate the confirmatory study as expeditiously as possible after aligning with the FDA on the trial design.
- In September 2026, the Company plans to present data from its ongoing Phase I/II studies of AMT-130. The update is expected to include follow-up data on all patients treated with AMT-130 in the first two cohorts, including four years of follow-up on 24 patients (12 patients at the high-dose and 12 patients at the low dose).
- In March 2026, the Company held a successful pre-submission meeting with the United Kingdom's (U.K.) Medicines and Healthcare products Regulatory Agency (MHRA) and the regulatory submission is progressing as planned for the third quarter of 2026.

Continued clinical progress in pipeline programs

AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (MTLE)

- In June 2026, the Company announced preliminary six-month follow-up data on the first, low dose cohort of six patients in the ongoing Phase I/IIa study.
    - As of the May 29, 2026 data cutoff date, three of six patients in the first, low-dose cohort achieved meaningful reductions in disabling seizures during months four through six of follow-up, ranging from a 79% to 100% decline from baseline. The remaining three patients in the low-dose cohort experienced variable changes in disabling seizures during months four through six of follow-up, ranging from a 33% decrease to a 36% increase compared to

baseline.
    - As of the June 19, 2026 presentation date, there were no Serious Adverse Events (SAEs) related to AMT-260 or the surgical procedure reported. All reported adverse events in the low dose cohort were classified as mild or moderate in severity, with the most common adverse event being headache (N=2). No immunosuppression was required.
- The Company expects to complete enrollment in the second dose cohort in the Phase I/IIa study in the third quarter of 2026.
- The Company expects to present updated results from the Phase I/IIa study in the first half of 2027.

AMT-191 for the treatment of Fabry disease

- In June 2026, the Company presented new data from the Phase I/II study of AMT-191 in Fabry disease. The data, with a cutoff date of March 15, 2026, included patient follow-up ranging from three months to more than 18 months and consisted of the following:
    - Dose-dependent elevations were observed across 11 patients in three dose levels with α-Gal A activity ranging from 1- to 16.2-fold above mean normal range (1.38-8.66 nmol; mean normal of 3.57 nmol) at the lowest dose, 14.5- to 229.6-fold at the mid dose, and 58.7- to 143.6-fold at the highest dose.
    - Plasma lyso-Gb3 levels remained stable post-dose across all cohorts, regardless of enzyme replacement therapy (ERT) status.
    - All 11 dosed patients were withdrawn from ERT.
    - AMT-191 continued to show a manageable safety profile at all dose levels. No SAEs related to AMT-191 were observed at the low and mid doses. No additional SAEs were observed at the high dose beyond those previously reported in September 2025 in two patients.
- Per protocol, additional dosing in the mid- and high-dose cohorts remains paused, pending further evaluation of asymptomatic liver enzyme elevations reported in two patients from the mid-dose cohort, which were confirmed as dose-limiting toxicities. These elevations resolved as of the end of May 2026 following a course of immunosuppression as per the study protocol.

Focused execution and strong financial position

- In June 2026, the Company closed an upsized underwritten public offering of 5,686,813 ordinary shares at a public offering price of $45.50 per share, including the full exercise of the underwriters' option to purchase additional shares. The aggregate gross proceeds to uniQure from the offering, before deducting the underwriting discounts and commissions and offering expenses payable by uniQure, were $259 million.
- As of June 30, 2026, the Company had cash, cash equivalents and current investment securities of $810.3 million. The Company expects that these resources will be sufficient to fund projected operating expenses into 2030.

Financial Highlights

Cash Position: As of June 30, 2026, the Company held $810.3 million in cash, cash equivalents and current investment securities, compared to $622.5 million as of December 31, 2025. The Company expects that these resources will be sufficient to fund our projected operating expenses into 2030.

Revenues: Revenue for the three months ended June 30, 2026 was $5.8 million, compared to $5.3 million in the same period in 2025. The increase of $0.5 million is due to an increase in license revenue, compared to the prior period.

R&D Expenses: Research and development expenses were $34.0 million for the three months ended June 30, 2026, compared to $35.4 million during the same period in 2025. The $1.4 million decrease was driven by a $3.2 million decrease in other research and development expenses, partially offset by a $1.8 million increase in direct research and development expenses. The decrease in other research and development expenses primarily reflected a $1.5 million decrease in facility expenses, a $1.3 million decrease in employee and contractor-related expenses, including share-based compensation, and a $1.0 million decrease in the fair value of contingent consideration, partially offset by a $0.6 million increase in information technology costs. The increase in direct research and development expenses reflected higher spend on the AMT-260, AMT-162 and AMT-191 programs, partially offset by lower spend on AMT-130, compared to the prior period.

SG&A Expenses: Selling, general and administrative expenses were $17.4 million for the three months ended June 30, 2026, compared to $13.5 million during the same period in 2025. The $3.9 million increase was primarily related to a $4.3 million increase in employee and contractor-related expenses, including share-based compensation, mainly as a result of a higher number of employees recruited in the second half of 2025 to support the potential commercial launches of AMT-130, a $0.7 million increase in intellectual property fees, and a $0.7 million increase in information technology costs and other expenses. This was partially offset by a $1.8 million decrease in professional fees, primarily as a result of lower costs incurred in support of the potential commercial launches of AMT-130, compared to the prior period.

Other Expense: Other expense increased to $8.0 million for the three months ended June 30, 2026 from $2.2 million during the same period in 2025, primarily due to a $6.0 million increase in costs associated with the supply of HEMGENIX® to CSL Behring.

Other non-Operating Items, net: Other non-operating items, net was an expense of $27.0 million for the three months ended June 30, 2026, compared to a gain of $6.6 million during the same period in 2025. The $33.6 million increase in net non-operating expense was primarily related to a $20.4 million unfavorable change in net foreign currency, from an $18.6 million gain to a $1.7 million loss, and a $16.0 million loss resulting from changes in the fair value of the liability related to pre-funded warrants, compared to nil in the prior period. This was partially offset by a $1.5 million increase in interest income and a $1.2 million decrease in interest expense, compared to the prior period.

Net loss: The net loss for the three months ending June 30, 2026, was $81.1 million, or $1.22 basic and diluted loss per ordinary share, compared to a $37.7 million net loss for the comparative period in 2025, or $0.69 basic and diluted loss per ordinary share.

Upcoming investor events:

- 2026 Biotech Summer Summit, August 10 -12 – Newport, RI

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Wednesday, July 29th 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. Analysts wishing to participate in the question and answer session should access the live call by dialing (646) 307-1963 or toll-free (800) 715-9871 and entering conference ID 5075555. If you are joining the conference call, please join 15 minutes before the start time.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. The approvals of uniQure's gene therapy for hemophilia B – an historic achievement based on more than a decade of research and clinical development – represent a major milestone in the field of genomic medicine and ushers in a new treatment approach for patients living with hemophilia. uniQure is now advancing a pipeline of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory temporal lobe epilepsy, 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. Examples of these forward-looking statements include, but are not limited to, statements concerning: the Company's cash runway and its ability to fund its operations into 2030; the Company's ability and plans to strategically advance its programs, including the potential commercialization of AMT-130; the Company's plans and timing with respect to future interactions with regulatory authorities and regulatory updates related to AMT-130; the Company's plans to conduct a confirmatory study for AMT-130, including the design, timing, endpoints and control arm of such study, and to align with the FDA on such study prior to BLA submission as well as the potential use of a concurrent standard-of-care control arm rather than a sham procedure; expectations regarding the timing of a BLA submission and receiving accelerated approval of AMT-130; the Company's plans to complete enrollment in the second dose cohort in the Phase I/IIa study for MTLE in the third quarter of 2026; the Company's plans to provide further clinical updates, including plans to announce topline four-year data from the Company's AMT-130 program in September 2026 and updated results from the Phase I/IIa study in MTLE in the first half of 2027; and the Company's plans to attend upcoming investor events. The Company's actual results could differ materially from those anticipated in these forward-looking statements for many reasons. These risks and uncertainties include, among others: risks associated with the clinical results and the development and timing of the Company's programs, including the risk that clinical results will be unable to demonstrate data sufficient to support further clinical development or regulatory approval in any country where approval is pursued; the risk that the FDA ultimately concludes that the Phase I/II trial data for AMT-130 are not sufficient to support a BLA or accelerated approval; the risk that more patient data become available that results in a different interpretation than the one derived from preliminary, interim or topline data; the Company's interactions with regulatory authorities, including the FDA and MHRA, which may affect the initiation, timing and progress of clinical trials and pathways and timing for regulatory approval; whether the measurements that the Company is evaluating are viewed as robust and sensitive measurements of disease progression suitable for regulatory approval; the Company's ability to conduct and fund any required confirmatory study for AMT-130; the Company's ability to successfully complete any required confirmatory study for AMT-130; the risk that accelerated approval, if granted, may be subject to post-approval requirements that are difficult or costly to satisfy; the Company's ability to continue to build and maintain the infrastructure and personnel needed to achieve its goals; the Company's effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of gene therapies; the Company's ability to demonstrate the therapeutic benefits of its gene therapy candidates in clinical trials; the Company's ability to obtain, maintain and protect intellectual property; and the Company's ability to fund its operations. These risks and uncertainties are more fully described under the heading "Risk Factors" in the Company's periodic filings with the U.S. Securities & Exchange Commission ("SEC"), including the Company's Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q, and in other filings that the Company makes with the SEC from time to time. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and, except as required by law, the Company assumes 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

	June 30, 2026	December 31, 2025
	(in thousands, U.S. dollars)	
Current assets		
Cash and cash equivalents	$ 413,027	$ 80,240
Current investment securities	397,308	542,301
Accounts receivable	5,778	5,863
Prepaid expenses	10,245	20,506
Other current assets and receivables	7,911	7,076
Total current assets	**834,269**	**655,986**
Non-current assets		
Property, plant and equipment, net	$ 11,376	$ 13,800
Other investments	30,267	30,237
Operating lease right-of-use assets	11,606	12,525
Intangible assets, net	62,101	72,790
Goodwill	24,587	25,355
Deferred tax assets, net	6,362	8,654
Other non-current assets	4,535	5,561
Total non-current assets	**150,834**	**168,922**
Total assets	**$ 985,103**	**$ 824,908**
Current liabilities		
Accounts payable	$ 4,979	$ 5,170
Accrued expenses and other current liabilities	51,133	41,292
Liability related to pre-funded warrants	24,242	12,595
Current portion of operating lease liabilities	2,991	3,862
Total current liabilities	**83,345**	**62,919**
Non-current liabilities		
Long-term debt	50,147	49,699
Liability from royalty financing agreement	489,333	473,199
Operating lease liabilities, net of current portion	9,696	9,832
Contingent consideration	18,113	18,736
Deferred tax liability, net	7,726	7,967
Other non-current liabilities, net of current portion	2,693	3,655
Total non-current liabilities	**577,708**	**563,088**
Total liabilities	**661,053**	**626,007**
Shareholders' equity		
Total shareholders' equity	**324,050**	**198,901**
Total liabilities and shareholders' equity	**$ 985,103**	**$ 824,908**

uniQure N.V.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2026	2025
	(in thousands, U.S dollars, except share and per share amounts)	
Total revenues	**$ 5,841**	**$ 5,262**
Operating expenses:		
Cost of license revenues	(350)	(656)
Research and development expenses	(33,964)	(35,383)
Selling, general and administrative expenses	(17,367)	(13,500)
Total operating expenses	**(51,681)**	**(49,539)**
Other income	1,598	2,597
Other expense	(7,961)	(2,185)
Loss from operations	**(52,203)**	**(43,865)**

Non-operating items, net	(27,015)	6,571
Loss before income tax expense	**$ (79,218)**	**$ (37,294)**
Income tax expense	(1,841)	(425)
Net loss	**$ (81,059)**	**$ (37,719)**
Basic and diluted net loss per ordinary share	$ (1.22)	$ (0.69)
Weighted average shares used in computing basic and diluted net loss per ordinary share	66,243,879	54,807,967

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