uniQure Announces Third Quarter 2026 Financial Results and Provides Company Update

November 4, 2026

~ FDA granted priority review of BLA for ifezuntirgene inilparvovec (AMT-130) in Huntington's disease; PDUFA target action date of May 1, 2027 ~

~ Aligned with FDA on key elements of confirmatory study design; U.S. patient screening expected to begin by end of 2026 ~

~ Cash, cash equivalents and investment securities of approximately $768.4 million as of September 30, 2026 expected to fund operations into 2030 ~

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

LEXINGTON, Mass. and AMSTERDAM, Nov. 04, 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 third quarter of 2026 and highlighted recent progress across its business.

"Over the last few months, we have made significant progress in our mission to potentially deliver the first disease-modifying therapy to the patients and families living with Huntington's disease," said Matthew Kapusta, chief executive officer at uniQure. "We strongly believe AMT-130 has the potential to deliver meaningful benefit to HD patients. With our BLA under Priority Review and preparations for the confirmatory study underway, we are committed to bringing this therapy to patients and families who continue to live with this devastating disease."

"As we continue to build our commercial capabilities and prepare for a potential launch, we are energized by what lies ahead, and our financial strength provides us the runway to deliver on our plans," Mr. Kapusta continued. "We are grateful to the patients, families and investigators who have made this progress possible, and are honored to continue to work alongside this community."

Recent Company Developments and Updates

Advancing ifezuntirgene inilparvovec towards potential accelerated approval for the treatment of Huntington's disease

- In November 2026, the U.S. Food and Drug Administration (FDA) accepted for review the Company's Biologics License Application (BLA) for ifezuntirgene inilparvovec, submitted under the Accelerated Approval pathway. The BLA was granted Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of May 1, 2027. The BLA is supported by 36-month data from 12 high-dose patients compared with a propensity score-matched external control.

- In September 2026, the Company announced additional data from its ongoing Phase I/II studies with a data cutoff of June 30, 2026.

    - In the 12 high-dose patients with 48 months of follow-up, composite Unified Huntington's Rating Scale (cUHDRS), the pre-specified primary endpoint for the analysis, showed a 44% slowing of disease progression versus the updated external control (non-significant p=0.144). Total Functional Capacity (TFC), the primary measure for the confirmatory study, showed a 61% slowing (nominal p=0.008) at 48 months. At 48-months, high-dose patients compared to low-dose patients showed 1.1 points less decline on cUHDRS and 0.33 points less decline on TFC, consistent with a dose-dependent treatment effect. Cerebrospinal fluid neurofilament light chain (NfL) remained near baseline at 48 months.

    - An updated 36-month analysis including all 15 high-dose patients showed an 80% slowing on cUHDRS (nominal p=0.005) and a 67% slowing on TFC (nominal p=0.011).

    - Ifezuntirgene inilparvovec was generally well tolerated with a manageable safety profile across 51 total treated patients.

- The Company and the FDA reached alignment on key elements of the confirmatory study of ifezuntirgene inilparvovec. The study is designed as a randomized, standard-of-care-controlled trial enrolling approximately 200 patients randomized in a 2:1 ratio to treatment or standard of care, with TFC as the primary endpoint at 36 months. The Company plans to initiate patient screening, beginning in the U.S., by the end of 2026.

- In the third quarter of 2026, the United Kingdom's (U.K.) Medicines and Healthcare products Regulatory Agency (MHRA) validated the Marketing Authorization Application (MAA) for ifezuntirgene inilparvovec, which was submitted to the MHRA earlier in the quarter.

- The Company continues to advance its launch readiness activities, including the build out of its field medical, market access, and strategic account teams as well as engagement with U.S. Huntington's disease treatment centers.

Continued clinical progress in pipeline programs

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

- In the third quarter of 2026, the Company completed enrollment in the second dose cohort of seven patients in the ongoing Phase I/IIa study and expects to present updated results in the first half of 2027.

AMT-191 for the treatment of Fabry disease

- In August 2026, enrollment resumed in the mid- and high-dose cohorts of the ongoing Phase I/II study of AMT-191 in Fabry disease. Per protocol, dosing had previously been paused after observing asymptomatic liver enzyme elevations in two mid-dose patients, which resolved following a course of immunosuppression.

- The Company expects to present updated results from the Phase I/II study in all dosing cohorts in the first half of 2027.

Focused execution and strong financial position

- As of September 30, 2026, the Company had cash, cash equivalents and investment securities of $768.4 million. The Company expects that these resources will be sufficient to fund projected operating expenses into 2030.

Financial Highlights

Cash Position: As of September 30, 2026, the Company held $768.4 million in cash, cash equivalents and investment securities, compared to $622.5 million as of December 31, 2025. The Company expects that these resources will be sufficient to fund projected operating expenses into 2030.

Revenues: Revenue for the three months ended September 30, 2026 was $5.2 million, compared to $3.7 million in the same period in 2025. The increase of $1.5 million is due to an increase in license revenue, compared to the prior period.

R&D Expenses: Research and development expenses were $35.5 million for the three months ended September 30, 2026, compared to $34.4 million in the same period in 2025. The $1.1 million increase primarily resulted from a $3.6 million increase in employee and contractor-related expenses including share-based compensation partially offset by a $2.3 million decrease in direct research and development expenses. The decrease in direct research and development expenses resulted from a $4.1 million reduction in process validation expenses related to ifezuntirgene inilparvovec, a $1.5 million increase in expenses related to AMT-260 and a $1.7 million increase in expenses related to preclinical activities and technology innovation projects.

SG&A Expenses: Selling, general and administrative expenses were $23.9 million for the three months ended September 30, 2026, compared to $19.4 million during the same period in 2025. The $4.5 million increase was primarily related to a $3.0 million increase in employee and contractor-related expenses, including share-based compensation, mainly as a result of the recruitment efforts over the last 12 to 15 months, a $0.7 million increase in professional fees and a $0.7 million increase in information technology costs to support the potential commercial launches of ifezuntirgene inilparvovec.

Non-Operating Items, net: Other non-operating items, net was an expense of $7.6 million for the three months ended September 30, 2026, compared to an expense of $20.9 million during the same period in 2025. The $13.3 million decrease in net non-operating expense was primarily due to $6.3 million reduction of expenses related to pre-funded warrants as well as $6.8 million improvement of net interest income.

Net loss: The net loss for the three months ending September 30, 2026, was $61.2 million, or $0.88 basic and diluted loss per ordinary share, compared to $80.5 million net loss for the comparative period in 2025, or $1.38 basic and diluted loss per ordinary share.

Upcoming investor events:

- Guggenheim 3rd Annual Healthcare Conference, November 10 – Boston, MA
- Stifel 2026 Healthcare Conference, November 11 – New York, NY
- UBS Global Biopharma Conference 2026 – November 16 – London, England

Investor Conference Call and Webcast Information

uniQure management will host an investor conference call and webcast today, Wednesday, November 4th 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; its belief related to the potential for ifezuntirgene inilparvovec, if approved, to meaningfully reduce disease progression based on the Company's evaluation and view of certain measurements of disease progression the Company's ability and plans to strategically advance its programs, including the potential commercialization of ifezuntirgene inilparvovec; the Company's future interactions with regulatory authorities and regulatory updates related to ifezuntirgene inilparvovec ; the Company's plans and ability to conduct a confirmatory study for ifezuntirgene inilparvovec , including the design, timing, endpoints and control arm of such study; ; 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; expectations regarding the adequacy of clinical data to support the marketing application and approval of ifezuntirgene inilparvovec; expectations regarding timing of receiving potential approval of ifezuntirgene inilparvovec; the risk that the FDA ultimately concludes that the Phase I/II trial data for ifezuntirgene inilparvovec are not sufficient to support approval of ifezuntirgene inilparvovec; 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 for the Company's other clinical candidates; the Company's ability to conduct and fund any required confirmatory study for ifezuntirgene inilparvovec ; the Company's ability to successfully complete any required confirmatory study for ifezuntirgene inilparvovec ; 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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UNAUDITED CONSOLIDATED BALANCE SHEETS

	September 30, 2026	December 31, 2025
	(in thousands, U.S. dollars)	
Current assets		
Cash and cash equivalents	$ 98,442	$ 80,240
Current investment securities	570,021	542,301
Accounts receivable	4,804	5,863
Prepaid expenses	9,601	20,506
Other current assets and receivables	9,677	7,076
Total current assets	**692,545**	**655,986**
Non-current assets		
Property, plant and equipment, net	$ 9,923	$ 13,800
Other investments	31,539	30,237

Non-current investment securities		99,967		- -
Operating lease right-of-use assets		10,980		12,525
Intangible assets, net		61,855		72,790
Goodwill		24,503		25,355
Deferred tax assets, net		5,709		8,654
Other non-current assets		3,530		5,561
Total non-current assets		**248,006**		**168,922**
Total assets	**$**	**940,551**	**$**	**824,908**
Current liabilities				
Accounts payable	$	6,173	$	5,170
Accrued expenses and other current liabilities		50,979		41,292
Liability related to pre-funded warrants		-		12,595
Current portion of operating lease liabilities		2,990		3,862
Total current liabilities		**60,142**		**62,919**
Non-current liabilities				
Long-term debt		50,345		49,699
Liability from royalty financing agreement		497,271		473,199
Operating lease liabilities, net of current portion		9,032		9,832
Contingent consideration		18,742		18,736
Deferred tax liability, net		7,699		7,967
Other non-current liabilities, net of current portion		2,667		3,655
Total non-current liabilities		**585,756**		**563,088**
Total liabilities		**645,898**		**626,007**
Shareholders' equity				
Total shareholders' equity		**294,653**		**198,901**
Total liabilities and shareholders' equity	**$**	**940,551**	**$**	**824,908**

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended September 30,			
	2026		**2025**	
	(in thousands, U.S dollars, except share and per share amounts)			
Total revenues	**$**	**5,196**	**$**	**3,701**
Operating expenses:				
Cost of license revenues		(236)		(398)
Research and development expenses		(35,517)		(34,366)
Selling, general and administrative expenses		(23,918)		(19,438)
Total operating expenses		**(59,671)**		**(54,202)**
Other income		1,690		1,510
Other expense		(195)		(2,044)
Loss from operations		**(52,980)**		**(51,035)**
Non-operating items, net		(7,561)		(20,868)
Loss before income tax expense	**$**	**(60,541)**	**$**	**(71,903)**
Income tax expense		(676)		(8,626)
Net loss	**$**	**(61,217)**	**$**	**(80,529)**
Basic and diluted net loss per ordinary share	$	(0.88)	$	(1.38)
Weighted average shares used in computing basic and diluted net loss per ordinary share		69,900,863		58,516,415

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