



## uniQure Announces 2023 Financial Results and Highlights Recent Company Progress

February 28, 2024

- ~ Presented promising clinical update from U.S. and European Phase I/II trials of AMT-130 in Huntington's disease; Up to three years of follow-up data to be presented in mid-2024; Regulatory interactions and clarity on potential strategies for clinical development expected in 2024 ~
- ~ Announced FDA clearance of two Investigational New Drug (IND) applications; Initiation of Phase I/II clinical trials in mesial temporal lobe epilepsy (mTLE) and Fabry disease, in addition to SOD1-ALS, are expected in the first half of 2024 ~
- ~ Announced strategic reorganization to advance multiple clinical-stage programs and deliver \$180 million of cost savings over the next three years ~
- ~ Cash position of approximately \$618 million as of December 31, 2023 expected to fund operations into the second quarter of 2027 ~

LEXINGTON, Mass. and AMSTERDAM, Feb. 28, 2024 (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 fourth quarter and full year 2023 and highlighted recent progress across its business.

"We are pleased with the progress made across the company in 2023 and are now laser-focused on execution across multiple clinical programs," stated [Matt Kapusta, chief executive officer of uniQure](#). "Our top priorities are to engage with regulatory authorities to clarify the approval pathway for AMT-130 in Huntington's disease and to initiate patient enrollment across three additional clinical trials as expeditiously as possible."

"While we enter 2024 with a strong balance sheet, we remain disciplined on the prudent and efficient allocation of our capital," he continued. "Any decision to advance AMT-130 into late-stage development will necessitate a clear and timely approval pathway and financial feasibility, including through a partnership which we would secure before beginning any Phase III trial."

### Recent Updates

- *Advancing AMT-130 for the treatment of Huntington's disease*
  - In December 2023, the Company announced updated clinical data from the ongoing U.S. and European Phase I/II studies of AMT-130 for the treatment of early-stage Huntington's disease.
    - Patients treated with AMT-130 continued to show evidence of preserved neurological function with potential dose-dependent clinical benefits relative to a non-concurrent criteria-matched natural history of the disease. When compared to the expected rate of decline from an inclusion criteria-matched natural history data set, AMT-130 showed favorable trends in composite Unified Huntington's Disease Rating Scale (cUHDRS), Total Functional Capacity (TFC) and Total Motor Score (TMS) key clinical rating scales designed to assess disease progression.
    - Further declines in neurofilament light chain (NfL), an exploratory biomarker for the measurement of neuronal degradation and disease progression were observed among patients treated with AMT-130. Mean NfL in the cerebrospinal fluid (CSF) for low-dose patients remained below baseline at 30 months of follow-up and high-dose patients were near baseline at 18 months.
    - AMT-130 continued to be generally well-tolerated with a manageable safety profile across both doses.
  - In the fourth quarter of 2023, the Company initiated patient dosing in a third cohort of up to 12 patients to further investigate both doses of AMT-130 in combination with perioperative immunosuppression, with a focus on evaluating near-term safety and tolerability. Enrollment in this cohort is expected to be completed in the second half of 2024.
  - In the second quarter of 2024, the Company expects to initiate regulatory interactions with the U.S. Food and Drug Administration (FDA) to discuss data from the ongoing Phase I/II studies and potential strategies for the further development of AMT-130. By the end of 2024, the Company expects to have greater clarity regarding a potential approval pathway for AMT-130.
  - In mid-2024, the Company expects to provide an interim update from the ongoing Phase I/II studies of AMT-130, including up to 24- and 36-month follow-up data from all treated patients in the U.S. and European trials.

- *Advancing additional programs into the clinic*
  - *AMT-260 for the treatment of refractory mesial temporal lobe epilepsy (rMTLE)* – In September 2023, the Company announced the clearance of an IND for the Phase I/IIa clinical study of AMT-260. Site initiation is underway, and patient enrollment is expected to begin in the first half of 2024.
  - *AMT-162 for the treatment of SOD1 amyotrophic lateral sclerosis (ALS)* - In January 2023, the Company entered into a global licensing agreement with Apic Bio for ABP-102, now known as AMT-162, for the treatment of superoxide dismutase 1 (SOD1) ALS, a rare, genetic form of ALS. Patient enrollment in a Phase I/II clinical trial is expected to begin in the first half of 2024.
  - *AMT-191 for the treatment of Fabry disease* – In November 2023, the Company announced the clearance of an IND for the Phase I/IIa clinical study of AMT-191. Patient enrollment is expected to begin in the first half of 2024.
- *Generating value from the commercial launch of HEMGENIX®*
  - The Company continues to provide commercial supply of etranacogene dezaparvovec (HEMGENIX®) to its partner CSL Behring and manufactures the product at its cGMP facility in Lexington, MA. The Company is one of a select number of gene therapy companies with a qualified facility producing routine commercial manufacturing for the market.

#### Upcoming Investor Events

- 44<sup>th</sup> Annual Cowen Health Care Conference, March 5, 2024 – Boston, MA
- Leerink Global Biopharma Conference 2024, March 12, 2024 – Miami, FL
- UBS Virtual CNS Day, March 18, 2024

#### Financial Highlights

**Cash position:** As of December 31, 2023, the Company held cash and cash equivalents and investment securities of \$617.9 million, compared to \$392.8 million as of December 31, 2022. The Company entered into a royalty agreement in May 2023 and received an upfront payment of \$375.0 million, and collected \$100.0 million in July 2023 for a milestone due from CSL following the first sale of HEMGENIX® in the U.S. The Company expects cash, cash equivalents and investment securities will fund operations into the second quarter of 2027.

**Revenues:** Revenue for the year ended December 31, 2023 was \$15.8 million, compared to \$106.5 million in the same period in 2022. Revenues in 2022 included \$100.0 million of license revenue related to the U.S. first sale milestone payment of HEMGENIX® that the Company expected to receive in 2023. Revenue from contract manufacturing HEMGENIX® for CSL increased \$9.1 million in 2023 compared to 2022.

**Cost of contract manufacturing revenues:** Cost of contract manufacturing revenues were \$13.6 million for the year ended December 31, 2023, compared to \$2.1 million for the same period in 2022. The increase relates to an increase in activities related to contract manufacturing HEMGENIX® for CSL.

**R&D expenses:** Research and development expenses were \$214.9 million for the year ended December 31, 2023, compared to \$197.6 million during the same period in 2022. The \$17.2 million increase was primarily related to a \$5.9 million net increase of external research and development expenses including a \$10.0 million payment made to Apic Bio to acquire AMT-162, an \$8.8 million non-cash increase related to the fair value of contingent consideration associated with the Company's acquisition of Corlieve Therapeutics in 2021 as well as a \$1.4 million non-cash impairment loss related to our Lexington research facility.

**SG&A expenses:** Selling, general and administrative expenses were \$74.6 million for the year ended December 31, 2023, compared to \$55.1 million during the same period in 2022. The \$19.5 million increase was primarily related to a \$9.7 million increase of professional, financial advisory and intellectual property fees, a \$3.7 million increase in information technology expenses as well as a \$3.7 million increase in personnel and contractor-related expenses.

#### Other non-operating items, net:

Other non-operating items, net was an expense of \$23.7 million for the year ended December 31, 2023, compared to net income of \$14.9 million for the same period in 2022. The \$38.6 million decrease in other non-operating items, net was primarily related to a decrease in foreign currency gains, net of \$24.9 million and an increase in non-cash interest expense of \$26.9 million related to the royalty agreement that the Company entered into in May 2023, which partially was offset by an increase of \$19.0 million in interest income earned on investment securities and cash on hand.

#### Net loss:

The net loss for the year ended December 31, 2023, was \$308.5 million, or \$6.47 basic and diluted loss per ordinary share, compared to \$126.8 million net loss for the same period in 2022, or \$2.71 basic and diluted loss per ordinary share.

#### About uniQure

uniQure's mission is to reimagine the future of medicine by delivering innovative cures that transform lives. The recent approvals of our gene therapy

for hemophilia B – a 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. We are now leveraging our modular and validated technology and manufacturing platform to advance a [pipeline](#) of proprietary gene therapies for the treatment of patients with Huntington's disease, refractory mesial temporal lobe epilepsy, amyotrophic lateral sclerosis (ALS), Fabry disease, and other severe diseases. [www.uniQure.com](http://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 the second quarter of 2027; the Company's expected cost savings related to its strategic reorganization; the Company's plans to announce additional follow up data from the ongoing U.S. and European Phase I/II clinical studies of AMT-130; the Company's plans to initiate interactions with regulatory authorities regarding the further development of AMT-130 and the timing of regulatory clarity from such interactions; and the Company's plans to initiate patient enrollment for AMT-260, AMT-162 and AMT-191 in the first half of 2024. 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; the Company's interactions with regulatory authorities, which may affect the initiation, timing and progress of clinical trials and pathways to approval; the Company's ability to continue to build and maintain the company 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; the Company's ability to fund its operations and to raise additional capital as needed; and the impact of global economic uncertainty, rising inflation, rising interest rates or market disruptions on its business. 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 its Annual Report on Form 10-K filed February 28, 2024 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 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

	December 31, 2023	December 31, 2022
	(in thousands, except share and per share amounts)	
<b>Current assets</b>		
Cash and cash equivalents	\$ 241,360	\$ 228,012
Current investment securities	376,532	124,831
Accounts receivable and contract asset	4,193	102,376
Inventories, net	12,024	6,924
Prepaid expenses	15,089	11,817
Other current assets and receivables	2,655	2,814
<b>Total current assets</b>	<b>651,853</b>	<b>476,774</b>
<b>Non-current assets</b>		
Property, plant and equipment, net	46,548	50,532
Non-current investment securities	—	39,984
Operating lease right-of-use assets	28,789	32,726
Intangible assets, net	60,481	58,778
Goodwill	26,379	25,581
Deferred tax assets, net	12,276	14,528
Other non-current assets	5,363	6,061
<b>Total non-current assets</b>	<b>179,836</b>	<b>228,190</b>
<b>Total assets</b>	<b>\$ 831,689</b>	<b>\$ 704,964</b>

<b>Current liabilities</b>			
Accounts payable	\$	6,586	\$ 10,984
Accrued expenses and other current liabilities	\$	30,534	\$ 30,571
Current portion of contingent consideration		28,211	25,982
Current portion of operating lease liabilities		8,344	8,382
<b>Total current liabilities</b>		<b>73,675</b>	<b>75,919</b>
<b>Non-current liabilities</b>			
Long-term debt		101,749	102,791
Liability from royalty financing agreement		394,241	—
Operating lease liabilities, net of current portion		28,316	31,719
Contingent consideration, net of current portion		14,795	9,334
Deferred tax liability, net		7,543	8,257
Other non-current liabilities		3,700	935
<b>Total non-current liabilities</b>		<b>550,344</b>	<b>153,036</b>
<b>Total liabilities</b>		<b>624,019</b>	<b>228,955</b>
<b>Shareholders' equity</b>			
<b>Total shareholders' equity</b>		<b>207,670</b>	<b>476,009</b>
<b>Total liabilities and shareholders' equity</b>	\$	<b>831,689</b>	\$ <b>704,964</b>
Balancing check		-	-

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

Years ended December 31,

	2023	2022	2021
	(in thousands, except share and per share amounts)		
License revenues	\$ 2,758	\$ 100,000	\$ 517,400
Contract manufacturing revenues	10,835	1,717	-
Collaboration revenues	2,250	4,766	6,602
<b>Total revenues</b>	<b>15,843</b>	<b>106,483</b>	<b>524,002</b>
<b>Operating expenses:</b>			
Cost of license revenues	(65)	(1,254)	(24,976)
Cost of contract manufacturing revenues	(13,563)	(2,089)	-
Research and development expenses	(214,864)	(197,591)	(143,548)
Selling, general and administrative expenses	(74,591)	(55,059)	(56,290)
<b>Total operating expenses</b>	<b>(303,083)</b>	<b>(255,993)</b>	<b>(224,814)</b>
Other income	6,059	7,171	12,306
Other expense	(1,690)	(820)	(876)
<b>(Loss) / income from operations</b>	<b>(282,871)</b>	<b>(143,159)</b>	<b>310,618</b>
Non-operating items, net	(23,686)	14,900	22,188
<b>(Loss) / income before income tax (expense) / benefit</b>	<b>\$ (306,557)</b>	<b>\$ (128,259)</b>	<b>\$ 332,806</b>
Income tax (expense) / benefit	(1,921)	1,470	(3,217)
<b>Net (loss) / income</b>	<b>\$ (308,478)</b>	<b>\$ (126,789)</b>	<b>\$ 329,589</b>
<b>Earnings per ordinary share - basic</b>			
Basic net (loss) / income per ordinary share	\$ (6.47)	\$ (2.71)	\$ 7.17
<b>Earnings per ordinary share - diluted</b>			
Diluted net (loss) / income per ordinary share	\$ (6.47)	\$ (2.71)	\$ 7.04
Weighted average shares - basic	47,670,986	46,735,045	45,986,467
Weighted average shares - diluted	47,670,986	46,735,045	46,840,972

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