



uniQure Announces Second Quarter 2021 Financial Results and Highlights Recent Company Progress

July 26, 2021

~ Expanded gene therapy pipeline with four new research programs and announced acquisition of Corlieve Therapeutics ~

~ Presented positive 52-week data from HOPE-B pivotal study demonstrating sustained increases in Factor IX (FIX) levels with mean FIX activity of 41.5 percent of normal and significant reductions in bleeding events ~

~ Completed its global commercialization and license transaction with CSL Behring for hemophilia B gene therapy program ~

~ Initiated patient enrollment in second dose cohort of Phase I/II clinical trial of AMT-130 for Huntington's disease, with initiation of a European Phase Ib/II clinical trial expected in second half of 2021 ~

LEXINGTON, Mass. and AMSTERDAM, The Netherlands, July 26, 2021 (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 2021 and highlighted recent progress across its business.

"We had a highly productive second quarter of this year marked by several notable achievements, including the closing on our global commercial licensing agreement with CSL Behring in hemophilia B and the recent presentation of 52-week data on all patients in our HOPE-B pivotal study showing durable and sustained increases in Factor IX levels within the non-hemophiliac range¹, as well as significant reductions in both bleeding events and usage of replacement therapy after a one-time administration of etranacogene dezaparvovec," stated [Matt Kapusta, chief executive officer at uniQure](#). "Additionally, we continued to make strong progress in our U.S. Phase I/II study of AMT-130 in Huntington's disease, including the completion of patient enrollment in the first dose cohort and the initiation of dosing in the second cohort."

"We also recently hosted a Research and Development Day where we disclosed four new promising research programs that complemented our expanding focus in neurological disease and larger patient populations, including the acquisition of Corlieve Therapeutics and its gene therapy program targeting temporal lobe epilepsy that builds on our expanding focus in serious neurological diseases," he added. "These investments deliver on our commitment to leverage our strong cash position to invest in new research programs and enabling technologies with the potential to drive significant value. In the second half of 2021, we expect to continue our progress across all programs, including availability of 78-week data from the HOPE-B study, initiating patient enrollment for our open-label European study of AMT-130 in Huntington's disease, and announcing initial data on the first four randomized Huntington's disease patients in the ongoing U.S. Phase I/II study."

¹ Srivastava et al, Haemophilia. 2020;26(Suppl 6):1-158.

Recent Company Progress

- *Advancing late-stage development of etranacogene dezaparvovec (AMT-061) for the treatment of hemophilia B*
 - In June 2021, the Company announced initial 52-week post-administration Factor IX data and annualized bleeding rates (ABR) in the [HOPE-B pivotal trial of etranacogene dezaparvovec](#) in severe to moderately severe hemophilia B patients. Sustained increase in FIX levels was observed with a mean FIX activity of 41.5% of normal compared to 39.0% at 26-weeks post administration. During the 52-week period after dosing, patients reported an 80% reduction in total bleeding events requiring treatment, an 85% reduction in spontaneous bleeding events requiring treatment, and a 96% reduction in annualized usage of FIX replacement therapy.
 - The Company expects all patients in the HOPE-B clinical trial to complete their 78-week follow-up visits by the end of the third quarter of 2021, and expects the BLA will be submitted in the first quarter of 2022.
 - The Company announced the closing of the transaction with CSL Behring for its hemophilia B gene therapy following the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 on May 5, 2021. In accordance with the agreement, the upfront payment of \$450 million was paid to uniQure on May 7, 2021.
- *Advancing the clinical development of AMT-130 for the treatment of Huntington's disease*
 - In May 2021, the Company announced the completion of patient enrollment of the first dose cohort of ten patients in its ongoing Phase I/II study of AMT-130 in Huntington's disease. The U.S. study is a multi-center, double-blinded, randomized and controlled trial to assess the safety and efficacy of AMT-130. The Data Safety Monitoring Board

recommended proceeding with patient enrollment in the second dose cohort after reviewing nine-month safety data from the first two enrolled patients, six-month safety data from the next two enrolled patients, and the 30-day safety data from the remaining six patients.

- In June 2021, the Company announced the first two patient procedures were completed in the second dose cohort of Phase I/II clinical trial. Enrollment of the next two patient procedures will take place after a planned DSMB meeting expected to be held later in the third quarter of 2021.
- Initial data from the first four enrolled patients in this first-in-human AAV-gene therapy trial are anticipated by year-end 2021. The Company also remains on track to initiate an open label, Phase Ib/II clinical study of AMT-130 in Europe with enrollment beginning in the second half of 2021.

- *Expanding the pipeline and strengthening the platform*

- In June, the Company announced the planned acquisition of Corlieve Therapeutics, a pre-clinical gene therapy company based in France. Corlieve's lead program, now designated as AMT-260, employs miRNA silencing technology to suppress aberrantly expressed kainite receptors in the hippocampus of patients with temporal lobe epilepsy (TLE). TLE represents a large unmet clinical need affecting approximately 1.3 million people in the U.S. and Europe alone, of which approximately 800,000 patients are unable to adequately control acute seizures with currently approved anti-epileptic therapies. The transaction is subject to customary closing conditions as well as review by the French Ministry of Economy, Finance and Recovery. The acquisition is expected to close in the third quarter of 2021.
- In June, the Company hosted a virtual Research & Development Day to highlight new investments in its expanding gene therapy pipeline focusing on central nervous system and rare, liver-directed disorders, as well as new advancements in platform technology and manufacturing. Three new, internally developed pre-clinical programs were announced in Parkinson's disease (AMT-210), amyotrophic lateral sclerosis (ALS) (AMT-160), and autosomal dominant Alzheimer's disease (AMT-240). Additional highlights from the event included updates on the clinical development of AMT-130 in Huntington's disease and advancements in the ongoing preclinical development of AMT-150 for spinocerebellar ataxia 3 and AMT-190 for Fabry disease.
- In February 2021, the Company began an expansion of the Amsterdam site to include a cleanroom capable of manufacturing cGMP materials at a 500-liter scale and additional laboratories to support the expansions of research and development activities.

- *Expansion of the leadership team*

- In May 2021, Pierre Caloz joined the leadership team as Chief Operating Officer overseeing all manufacturing operations, global CMC development and innovation, supply chain and facilities. Mr. Caloz joins the Company with nearly 20 years of global operations experience in the biopharma industry, including CSL Behring, Merck-Serono, Abgenix and Amgen.
- On June 15, 2021, Christian Klemt was appointed as Chief Financial Officer. Mr. Klemt was the Company's Chief Accounting Officer from August 2017 to June 2021, and he will continue to serve as general manager of the Amsterdam site.

- *Strong cash position to advance the Company's programs*

- As of June 30, 2021, the Company's cash position was \$677.3 million. The Company expects cash and cash equivalents will be sufficient to fund operations into the first half of 2024, excluding any of the \$1.6 billion in future milestones or royalties on net sales the Company may receive under the CSL Behring Agreement. The receipt of approximately \$300 million in milestone payments relates to regulatory submissions, first commercial sales and certain development milestones would be expected to extend the cash runway by approximately 18 months.

Upcoming Investor Events (each to be conducted virtually)

- Citi's Annual Biopharma Virtual Conference, September 8-10, 2021
- Wells Fargo Virtual Healthcare Conference, September 9-10, 2021
- SVB Leerink CybeRx Series Event, September 21-23, 2021
- Cantor Fitzgerald Virtual Global Healthcare Conference, September 27-29, 2021
- Chardan's 5th Annual Genetic Medicines Conference, October 4-5, 2021

Financial Highlights

Cash Position: As of June 30, 2021, the Company held cash and cash equivalents of \$677.3 million, compared to \$244.9 million as of December 31, 2020. Upon the CSL Behring Agreement becoming fully effective on May 6, 2021, the Company received \$462.4 million of payments. Additionally, in January 2021, the Company and Hercules entered into an amended debt facility agreement increasing the aggregate principle to up to \$135.0 million, of which the Company drew down an additional \$35.0 million for a total of \$70.0 million outstanding under the facility as of June 30, 2021. In March 2021, the Company entered into an Open Market Sale Agreement with SVB Leerink LLC providing for the sale of up to \$200.0 million of its ordinary shares from time to time in 'at-the-market' offerings under a shelf registration statement. Pursuant to this agreement, as of April 30, 2021, the Company had sold 921,730 ordinary shares for gross proceeds of approximately \$29.6 million.

Revenues: Revenue for the three months ended June 30, 2021, were \$463.9 million, compared to \$1.5 million during the same period in 2020. The increase is a result of \$462.4 million of license revenue recognized from the CSL Behring Agreement.

Cost of Contract Revenues: Cost of contract revenues for the three months ended June 30, 2021, was \$23.2 million compared to nil for the same period in 2020. Costs incurred in 2021 are associated with the license revenue recognized under the CSL Behring Agreement.

R&D Expenses: Research and development expenses were \$32.8 million for the three months ended June 30, 2021, compared to \$28.4 million during the same period in 2020. The change was primarily related to recruitment of personnel to support the development of product candidates, advancing the clinical developments of Huntington's disease, and increased activities associated with preclinical product candidates.

SG&A Expenses: Selling, general and administrative expenses were \$17.3 million for the three months ended June 30, 2021, compared to \$11.5 million during the same period in 2020. The change was primarily related to costs incurred for financial advisory fees in relation to the licensing transaction with CSL Behring, increases in personnel and consulting expenses to support growth, and increased share-based compensation.

Other non-operating items, net: Other income, net was \$4.7 million for the three months ended June 30, 2021, compared to other expense, net of \$4.3 million during the same period in 2020. The increase in income is primarily due to foreign currency gains in the current period compared to a net foreign currency loss in the same period in 2020.

Net Loss: The net income for the three months ended June 30, 2021, was \$399.5 million, or \$8.68 basic net income per share and \$8.51 diluted net income per share, compared to a loss of \$42.6 million, or \$0.96 basic and diluted loss per share during the same periods in 2020.

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 gene therapies to treat patients with hemophilia B, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other 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, uniQure's expectations its plans to announce data from the HOPE-B pivotal trial, patient enrollment in the second cohort of AMT-130, the initiation of an open-label European study of AMT-130, the announcement of data on the first four patients in the Phase I/II study of AMT-130, the submission of the regulatory filing for marketing approval of etranacogene dezaparvovec, the potential financial compensation we may be paid pursuant to our agreement with CSL Behring, or other updates on the research pipeline, and our plans to advance or expand our pipeline, accelerate research, identify business development opportunities, invest in technology, or scale our manufacturing capabilities. uniQure'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 ongoing COVID-19 pandemic 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 uniQure's periodic securities filings, including its Annual Report on Form 10-K filed March 1, 2021. 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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UNAUDITED CONSOLIDATED BALANCE SHEETS

June 30,
2021

December 31,
2020

(in thousands, except share and per share amounts)

Current assets		
Cash and cash equivalents	\$ 677,330	\$ 244,932
Accounts receivables	2,705	6,618
Prepaid expenses	10,861	4,337
Other current assets and receivables	3,328	3,024
Total current assets	694,224	258,911
Non-current assets		
Property, plant and equipment, net	38,371	32,328
Operating lease right-of-use assets	26,782	26,086
Intangible assets, net	2,637	3,361
Goodwill	525	542
Deferred tax assets	15,965	16,419
Other non-current assets	5,760	2,748
Total non-current assets	90,040	81,484
Total assets	\$ 784,264	\$ 340,395
Current liabilities		
Accounts payable	\$ 16,817	\$ 3,772
Accrued expenses and other current liabilities	23,526	18,038
Current portion of operating lease liabilities	5,752	5,524
Total current liabilities	46,095	27,334
Non-current liabilities		
Long-term debt	70,780	35,617
Operating lease liabilities, net of current portion	30,534	30,403
Other non-current liabilities	3,247	3,136
Total non-current liabilities	104,561	69,156
Total liabilities	\$ 150,656	\$ 96,490
Shareholders' equity		
Total shareholders' equity	633,608	243,905
Total liabilities and shareholders' equity	\$ 784,264	\$ 340,395

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UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

	Three months ended June 30,	
	2021	2020
	(in thousands, except share and per share amounts)	
Total revenues	\$ 463,868	\$ 1,535
Operating expenses:		
Cost of contract revenues	(23,178)	-
Research and development expenses	(33,401)	(28,401)
Selling, general and administrative expenses	(16,645)	(11,511)
Total operating expenses	(73,224)	(39,912)
Other income	7,590	669
Other expense	(226)	(500)
Loss from operations	398,008	(38,208)
Non-operating items, net	4,718	(4,343)
Loss before income tax expense	402,726	(42,551)
Income tax expense	(3,258)	-
Net loss	\$ 399,468	\$ (42,551)
Earnings per ordinary share - basic		
Basic net income/(loss) per ordinary share	\$ 8.68	\$ (0.96)
Earnings per ordinary share - diluted		
Diluted net income/(loss) per ordinary share	\$ 8.51	\$ (0.96)
Weighted average shares - basic	46,037,900	44,387,463
Weighted average shares - diluted	46,929,870	44,387,463