

**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 13, 2023**

**ORCHARD THERAPEUTICS PLC**

(Exact name of Registrant as Specified in Its Charter)

**England and Wales**

(State or Other Jurisdiction  
of Incorporation)

**001-38722**

(Commission File Number)

**Not Applicable**

(IRS Employer  
Identification No.)

**245 Hammersmith Road**

**London W6 8PW**

**United Kingdom**

(Address of Principal Executive Offices; Zip Code)

Registrant's Telephone Number, Including Area Code: **+44 (0) 203 808 8286**

**Not Applicable**

(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:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
American Depositary Shares, each representing ten ordinary shares, nominal value £0.10 per share	ORTX	The Nasdaq Capital Market

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

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.

## **Item 2.02 Results of Operations and Financial Condition.**

On November 13, 2023, Orchard Therapeutics plc (“Orchard”) announced its financial results for the quarter ended September 30, 2023 and other business updates. A copy of the press release is furnished as Exhibit 99.1 to this current report on Form 8-K.

### **Additional Information and Where to Find It**

In connection with the proposed transaction between Kyowa Kirin Co., Ltd. (“Kyowa Kirin”) and Orchard, Orchard has filed with the Securities and Exchange Commission (the “SEC”) a Proxy Statement, the definitive version of which (if and when available) will be mailed to Orchard security holders. Orchard may also file other documents with the SEC regarding the proposed transaction. This document is not a substitute for the Proxy Statement or any other document which Orchard may file with the SEC. INVESTORS AND SECURITY HOLDERS ARE URGED TO READ THE PROXY STATEMENT (WHICH WILL INCLUDE AN EXPLANATORY STATEMENT IN RESPECT OF THE SCHEME OF ARRANGEMENT OF ORCHARD, IN ACCORDANCE WITH THE REQUIREMENTS OF THE U.K. COMPANIES ACT 2006) AND ANY OTHER RELEVANT DOCUMENTS THAT ARE FILED OR WILL BE FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THESE DOCUMENTS, CAREFULLY AND IN THEIR ENTIRETY IF AND WHEN THEY BECOME AVAILABLE BECAUSE THEY CONTAIN OR WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION AND RELATED MATTERS. Investors and security holders may obtain a free copy of the Proxy Statement and other relevant documents containing important information about Kyowa Kirin, Orchard and the proposed transaction (if and when they become available) once such documents are filed with the SEC at the SEC’s website at [www.sec.gov](http://www.sec.gov). Copies of the documents filed with the SEC by Orchard will be available free of charge on Orchard’s website at [ir.orchard-tx.com](http://ir.orchard-tx.com) or by contacting Orchard’s Investor Relations Department at [investors@orchard-tx.com](mailto:investors@orchard-tx.com).

### **Participants in the Solicitation**

Orchard and certain of its directors and executive officers may be deemed to be participants in the solicitation of proxies in respect of the proposed transaction. Information regarding Orchard’s directors and executive officers, including a description of their direct interests, by security holdings or otherwise, is contained in Orchard’s proxy statement for its 2023 annual general meeting of shareholders, which was filed with the SEC on April 27, 2023, and subsequent statements of beneficial ownership on file with the SEC. Orchard shareholders may obtain additional information regarding the direct and indirect interests of the participants in the solicitation of proxies in connection with the proposed transaction, including the interests of Orchard directors and executive officers in the transaction, which may be different than those of Orchard shareholders generally, by reading the Proxy Statement if and when it is filed with the SEC and any other relevant documents that are filed or will be filed with the SEC relating to the transaction. You may obtain free copies of these documents using the sources indicated above.

### **Cautionary Statement Regarding Forward-Looking Statements**

This communication contains “forward-looking statements” within the meaning of the federal securities laws, including Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Exchange Act. These forward-looking statements are based on Orchard’s current expectations, estimates and projections about the expected date of closing of the proposed transaction and the potential benefits thereof, its business and industry, management’s beliefs and certain assumptions made by Orchard and Kyowa Kirin, all of which are subject to change. In this context, forward-looking statements often address expected future business and financial performance and financial condition, and often contain words such as “expect,” “anticipate,” “intend,” “plan,” “believe,” “could,” “seek,” “see,” “will,” “may,” “would,” “might,” “potentially,” “estimate,” “continue,” “expect,” “target,” “explore,” “evaluate,” “predict,” “project,” similar expressions or the negatives of these words or other comparable terminology that convey uncertainty of future events or outcomes. All forward-looking statements by their nature address matters that involve risks and uncertainties, many of which are beyond Orchard’s or Kyowa Kirin’s control, and are not guarantees of future results, such as statements about the consummation of the proposed transaction and the anticipated benefits thereof. These and other forward-looking statements, are not guarantees of future results and are inherently subject to risks, uncertainties and assumptions that could cause actual results to differ materially from those expressed in any forward-looking statements. Accordingly, there are or will be important factors that could cause actual results to differ materially from those indicated in such statements and, therefore, you should not place undue reliance on any such statements and caution must be exercised in relying on forward-looking statements. Important risk factors and uncertainties that may cause such a difference include, but are not limited to, risks and uncertainties surrounding: (i) the completion of the proposed transaction on anticipated terms and timing, including in connection with obtaining shareholder and regulatory approvals, the sanction of the High Court of Justice of England and Wales, satisfaction of other closing conditions to consummate the acquisition, anticipated tax treatment, unforeseen liabilities, future capital expenditures, revenues, expenses, earnings, synergies, economic performance, indebtedness, financial condition, losses, future prospects, business and management strategies for the management, expansion and growth of Orchard’s business and other conditions to the completion of the transaction; (ii) the occurrence of any event, change or other circumstance that could give rise to the termination of the definitive transaction agreement relating to the proposed transaction; (iii) Orchard’s ability to implement its business model and strategic plans for its product, product candidates and pipeline, and challenges inherent in developing, commercializing, manufacturing, launching, marketing and selling existing and new products; (iv) significant transaction costs associated with the proposed transaction; (v) potential litigation relating to

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the proposed transaction; (vi) the risk that disruptions from the proposed transaction will harm Orchard's business, including current plans, operations and collaborations, and including as a result of diverting the attention of Orchard's and Kyowa Kirin's management from ongoing business operations; (vii) the ability of Orchard to retain and hire key personnel; (viii) potential adverse reactions or changes to business relationships resulting from the announcement or completion of the proposed transaction; (ix) legislative, regulatory and economic developments affecting Orchard's business; (x) general economic and market developments and conditions; (xi) the evolving legal, regulatory and tax regimes under which Orchard operates; (xii) potential business uncertainty, including changes to existing business relationships, during the pendency of the transaction that could affect Orchard's financial performance; (xiii) restrictions during the pendency of the proposed transaction that may impact Orchard's ability to pursue certain business opportunities or strategic transactions; (xiv) the risk that Orchard may be unable to obtain governmental and regulatory approvals required for the proposed transaction, or that required governmental and regulatory approvals may delay the consummation of the proposed transaction or result in the imposition of conditions that could reduce the anticipated benefits from the proposed transaction or cause the parties to abandon the proposed transaction; (xv) unpredictability and severity of catastrophic events, including, but not limited to, global pandemic, acts of terrorism or outbreak of war or hostilities, as well as Orchard's response to any of the aforementioned factors; (xvi) potential delays or failures related to research, clinical trials and/or development of Orchard's programs or product candidates, which are based on novel gene therapy and (xvii) the risks related to non-achievement of the CVR milestone and that holders of the CVRs will not receive payments in respect of the CVRs. Additional factors that may affect the future results of Orchard are set forth in Orchard's filings with the SEC, including Orchard's most recently filed Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the SEC, which are available on the SEC's website at [www.sec.gov](http://www.sec.gov). See in particular Item 1A of Orchard's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 and Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2023 under the headings "Risk Factors." The risks and uncertainties described above and in the SEC filings cited above are not exclusive and further information concerning Orchard and its business, including factors that potentially could materially affect Orchard's business, financial conditions or operating results, may emerge from time to time. Moreover, other risks and uncertainties of which Orchard is not currently aware may also affect Orchard's forward-looking statements and may cause actual results and the timing of events to differ materially from those anticipated. Readers are urged to consider these factors carefully in evaluating these forward-looking statements, and not to place undue reliance on any forward-looking statements, which speak only as of the date hereof and reflect the views stated therein with respect to future events as at such dates, even if they are subsequently made available by Orchard on its website or otherwise. Readers should also carefully review the risk factors described in other documents that Orchard files from time to time with the SEC. Except as required by law, Orchard assumes no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

#### **No Offer or Solicitation**

This communication is not intended to and shall not constitute an offer to buy or sell or the solicitation of an offer to buy or sell any securities, or a solicitation of any vote or approval, nor shall there be any offer, solicitation or sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offer of securities shall be made in the United States absent registration under the U.S. Securities Act of 1933, as amended, or pursuant to an exemption from, or in a transaction not subject to, such registration requirements.

*The information contained in this Current Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.*

#### **Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits.

<b>Exhibit Number</b>	<b>Description</b>
99.1	<a href="#">Press release, dated November 13, 2023</a>
104	Cover page interactive data file (embedded within the Inline XBRL document)

**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 hereunto duly authorized.

**ORCHARD THERAPEUTICS PLC**

Date: November 13, 2023

By: /s/ Frank E. Thomas

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Frank E. Thomas

President and Chief Operating Officer

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**Orchard Therapeutics Reports Third Quarter 2023 Financial Results and Highlights Recent Business Accomplishments**

*Reported \$5.6 million in quarterly Libmeldy net revenue and \$12.7 million year-to-date*

*Planned acquisition by Kyowa Kirin valued at up to \$477.6 million with an anticipated closing in the first quarter of 2024*

*Biologics License Application for OTL-200 in MLD accepted by U.S. FDA under Priority Review; PDUFA date set for March 18, 2024*

*Ten prospective newborn screening studies are active globally with approximately 200,000 babies screened*

*Eight presentations at ESGCT highlighted the differentiated profile and potential broad applicability of the company's HSC gene therapy platform*

BOSTON and LONDON, November 13, 2023, (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced several business accomplishments along with its financial results for the quarter ended September 30, 2023.

"With strong execution across our key commercial, regulatory and clinical priorities, 2023 has been a transformational year for Orchard Therapeutics, culminating with the announcement of the planned acquisition by Kyowa Kirin," said Bobby Gaspar, M.D., Ph.D., chief executive officer. "As we enter this next phase in the company's evolution, we look forward to collaborating with our new colleagues after closing to fully unlock the curative potential of HSC gene therapy for the benefit of patients and society."

Dr. Gaspar continued, "With our BLA file for OTL-200 in MLD accepted by the FDA under Priority Review, we are one step closer to potentially bringing this important therapy to families in the U.S. Due to the nature of the disease and the urgency to treat children affected by MLD, we are working diligently in parallel to prepare for a potential launch in 2024 and ensure OTL-200 will be available to patients in the U.S. as quickly as possible. In addition, we remain well-positioned to continue investing in initiatives aimed at accelerating commercial growth in Europe and advancing our next-in-line neurometabolic programs in MPS disorders. The next 12 months have the potential to provide Orchard Therapeutics with several breakout opportunities that we believe would cement our leadership position in the HSC gene therapy field."

**Planned Acquisition of Orchard Therapeutics by Kyowa Kirin**

Last month, the company entered into a definitive agreement under which Kyowa Kirin will acquire Orchard Therapeutics, subject to certain closing conditions. Under the terms of the agreement, Kyowa Kirin will initiate a scheme of arrangement to acquire all outstanding shares of Orchard Therapeutics at a price of \$16.00 per American Depositary Share (ADS) in cash (or an aggregated value of approximately \$387.4 million) at closing, which represented a premium of 144% to Orchard Therapeutics' volume-weighted average price per ADS over the 30 days ended October 4, 2023, the day before the transaction was announced.

In connection with the transaction, an additional contingent value right (CVR) of \$1.00 per ADS is payable for a total of \$17.00 per ADS, or an aggregated total transaction value of approximately \$477.6 million, which includes certain warrants to purchase Orchard shares. The CVR payments are contingent and payable only on U.S. approval of OTL-200 in 2024 per the terms of the CVR agreement.

The transaction has been unanimously approved by both company's Boards of Directors and is expected to close in the first quarter of 2024 subject to Orchard Therapeutics' shareholder approval, receipt of applicable regulatory approvals and other customary closing conditions. A preliminary proxy statement has been filed with the U.S. Securities and Exchange Commission.

Following the completion of the acquisition, Orchard Therapeutics will become a wholly-owned subsidiary of Kyowa Kirin.

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## **BLA for OTL-200 in MLD Accepted by FDA Under Priority Review**

In September, the U.S. Food and Drug Administration (FDA) accepted the filing of the company's Biologics License Application (BLA) for OTL-200 in metachromatic leukodystrophy (MLD) under Priority Review. The agency has set a Prescription Drug User Fee Act (PDUFA) goal date of March 18, 2024.

OTL-200 previously received both Rare Pediatric Disease (RPD) and Regenerative Medicine Advanced Therapy (RMAT) designations from the FDA and is approved as Libmeldy® (atidarsagene autotemcel) by the European Commission (EC) and UK Medicines and Healthcare products Regulatory Agency (MHRA).

## **Commercial Updates and Newborn Screening Progress**

- In October, the Institute for Clinical and Economic Review (ICER), an independent non-profit research organization that analyzes the evidence on the effectiveness and value of medical treatments and services, published its Final Evidence Report on OTL-200.
  - ICER has determined an estimated value-based price benchmark for OTL-200 to be between \$2.3 to \$3.9 million. The upper end of the range is the highest for any therapy ICER has ever assessed and is based on a cost-effectiveness calculation at the threshold of \$150,000 per equal-value life-year gained.
  - Moreover, ICER gave OTL-200 the highest possible evidence rating with high certainty of a substantial net health benefit ("A") for the pre-symptomatic late infantile and pre-symptomatic early juvenile populations and better than average with moderate certainty of a substantial net health benefit versus usual care ("B+") for the early-symptomatic early juvenile subgroup.
  - In its totality, the Final Evidence Report provides additional support for the company's payer and reimbursement discussions in the U.S. and around the world.
- As the company continues to prepare for the potential launch of OTL-200, it is building out its regional team beginning with the appointment of Bennett Smith as senior vice president and general manager of North America.
  - Mr. Smith brings more than two decades of biopharmaceutical experience to Orchard Therapeutics, having served in leadership roles encompassing all commercial functions, including marketing, sales, market access and patient services. Prior to joining Orchard, Mr. Smith was senior vice president of commercial at Akebia Therapeutics. In this role, he served as an accomplished launch leader who simultaneously drove revenue growth for Akebia's in-line asset following the integration with Keryx Biopharmaceuticals.
- Ten prospective newborn screening studies are active throughout Europe, the U.S. and the Middle East, with approximately 200,000 babies screened.
  - To date, four confirmed cases of MLD have been identified through these studies. Eligible patients identified through newborn screening in Europe will continue to be treated commercially with Libmeldy in 2023 and beyond, adding to the referrals being generated through early symptomatic diagnosis and family screening.
  - The data from these studies will provide critical evidence to support applications for universal screening of MLD in the U.S. and around the world.
  - Furthering those efforts, the Illinois state legislature passed the Newborn Metabolic Screening Act, also known as SB67, which requires the state Department of Public Health to screen all newborns for MLD. The bill was signed by the governor in August, and it is expected Illinois will start the process of implementing statewide screening for MLD this year.

## **Recent Data Presentations**

Eight presentations highlighting the differentiated profile and potential broad applicability of the company's hematopoietic stem cell (HSC) gene therapy platform were featured last month at the European Society of Gene and Cell Therapy (ESGCT) 30th Annual Congress in Brussels.

Data highlights included:

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- A range of interim clinical outcomes in addition to the biochemical, neurological and skeletal results previously reported from the company's proof-of-concept study of OTL-203 in the Hurler subtype of mucopolysaccharidosis type I (MPS-IH). These new data demonstrate favorable outcomes for disease manifestations not effectively addressed by the current standard of care.
- A presentation supporting pre-clinical efficacy of OTL-204 which showed the ability of HSC gene therapy to restore microglial function, modulate neuroinflammation, and normalize predictive biomarkers in the progranulin form of frontotemporal dementia (GRN-FTD).
- A first look at pre-clinical data demonstrating the use of vectorized HSCs as a delivery vehicle for monoclonal antibodies.
- Additional abstracts from the company's pre-clinical research programs showing the therapeutic potential of HSC gene therapy to address larger indications, including a genetic sub-type of Crohn's disease and chronic autoimmune disorders.

In August, the company also presented data comprising the clinical package for the OTL-200 BLA in MLD at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in Jerusalem. Results showed that the administration of one-time gene therapy resulted in statistically significant improvement in severe motor impairment-free survival with up to 12 years of follow-up (median 6.76 years).

### Remaining 2023 Expected Milestones

Orchard Therapeutics has outlined the following key milestones expected for the remainder of 2023:

- Libmeldy for MLD: Continue to establish additional qualified treatment centers and expand newborn screening activities throughout Europe and the Middle East.
- OTL-200 for MLD: Prepare for potential U.S. launch, including establishing qualified treatment centers and expanding newborn screening activities in the country, in the first half of 2024 following an anticipated FDA decision on March 18, 2024.
- OTL-203 for MPS-IH: Initiate a global, multi-center registrational trial by year end.
- OTL-104 for NOD2-Crohn's disease: Commence IND- and CTA-enabling studies by year end, ahead of a potential filing in 2025.
- Advance the company's other pre-clinical programs, which includes OTL-204 in the progranulin form of FTD and OTL-105 partnered with and funded by Pharming Group N.V. in hereditary angioedema (HAE).

### Third Quarter 2023 Financial Results

Total revenue was \$6.3 million for the three months ended September 30, 2023, comprising \$5.6 million in Libmeldy revenue and \$0.7 million in collaboration revenue. This compares to total revenue of \$5.8 million in the same period in 2022, comprising \$5.4 million in Libmeldy revenue and \$0.4 million in collaboration revenue. The cost of product sales, which includes the cost of manufacturing, royalties to third parties and non-cash amortization, was \$1.7 million during the third quarter of 2023 compared to \$1.6 million in the same period in 2022. The company reported gross margins of approximately 72% for the three months ended September 30, 2023, consistent with previous quarters.

For the three months ended September 30, 2023, the company reported research and development (R&D) expenses of \$14.6 million, compared to \$18.1 million in the same period in 2022, a decrease of 19%. The decline was primarily the result of the release of the Strimvelis loss provision (\$2.9 million) following the previously announced European marketing authorization transfer to the therapy's originator.

For the three months ended September 30, 2023, the company reported selling, general and administrative (SG&A) expenses of \$11.6 million consistent with the \$11.5 million reported in the same period in 2022.

Loss from operations was \$21.6 million in the three months ended September 30, 2023, compared to a loss from operations of \$25.5 million in the corresponding period of 2022, a decrease of 15%. The decline was primarily the result of a \$3.5 million decrease in R&D expenses.

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Total other income (loss) was \$12.7 million for the three months ended September 30, 2023. The company reported an \$8.8 million loss relating to the fair value remeasurement of warrants that were issued in connection with the first and second closings of the strategic financing entered into in March 2023. The outstanding warrants will continue to be remeasured in future periods resulting in non-cash gains or losses based on a number of valuation assumptions on the underlying financial instruments. In addition, the U.S. dollar has strengthened against the British pound and Euro throughout the third quarter of 2023, resulting in unrealized losses on certain intercompany balances denominated in currencies other than the U.S. dollar.

Net loss was \$35.3 million for the three months ended September 30, 2023, compared to \$47.6 million in the same period in 2022, a reduction of 26%. The company had approximately 227.3 million ordinary shares, equivalent to 22.7 million American Depositary Shares, outstanding as of September 30, 2023.

As of September 30, 2023, the company reported cash, cash equivalents and investments of approximately \$125.4 million, with \$25.6 million of debt outstanding, compared to \$148.0 million and \$32.4 million of debt outstanding as of December 31, 2022. As a result of the anticipated acquisition by Kyowa Kirin, Orchard Therapeutics has removed its financial guidance around Libmeldy revenue and anticipated cash runway. The company expects that its existing cash, cash equivalents and investments will fund its anticipated operating, debt service and capital expenditure requirements for at least twelve months from the date of the filing of its Form 10-Q for the quarter ended September 30, 2023.

#### **About Libmeldy / OTL-200**

Libmeldy® (atidarsagene autotemcel), also known as OTL-200, has been approved by the European Commission for the treatment of MLD in eligible early-onset patients characterized by biallelic mutations in the ARSA gene leading to a reduction of the ARSA enzymatic activity in children with i) late infantile or early juvenile forms, without clinical manifestations of the disease, or ii) the early juvenile form, with early clinical manifestations of the disease, who still have the ability to walk independently and before the onset of cognitive decline. Libmeldy is the first therapy approved for eligible patients with early-onset MLD.

The most common adverse reaction attributed to treatment with Libmeldy was the occurrence of anti-ARSA antibodies. In addition to the risks associated with the gene therapy, treatment with Libmeldy is preceded by other medical interventions, namely peripheral blood mobilization and apheresis, followed by myeloablative conditioning, which carry their own risks. During the clinical studies of Libmeldy, the safety profiles of these interventions were consistent with their known safety and tolerability.

For more information about Libmeldy, please see the Summary of Product Characteristics (SmPC) available on the European Medicines Agency (EMA) website.

Libmeldy is approved in the European Union, UK, Iceland, Liechtenstein and Norway. OTL-200 is an investigational therapy in the U.S.

Libmeldy was developed in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

#### **About Orchard Therapeutics**

At Orchard Therapeutics, our vision is to end the devastation caused by genetic and other severe diseases. We aim to do this by discovering, developing and commercializing new treatments that tap into the curative potential of hematopoietic stem cell (HSC) gene therapy. In this approach, a patient's own blood stem cells are genetically modified outside of the body and then reinserted, with the goal of correcting the underlying cause of disease in a single treatment.

In 2018, the company acquired GSK's rare disease gene therapy portfolio, which originated from a pioneering collaboration between GSK and the San Raffaele Telethon Institute for Gene Therapy in Milan, Italy. Today, Orchard is advancing a pipeline spanning pre-clinical, clinical and commercial stage HSC gene therapies designed to address serious diseases where the burden is immense for patients, families and society and current treatment options are limited or do not exist.

Orchard has its global headquarters in London and U.S. headquarters in Boston. For more information, please visit [www.orchard-tx.com](http://www.orchard-tx.com), and follow us on Twitter and LinkedIn.

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### **Availability of Other Information About Orchard**

Investors and others should note that Orchard communicates with its investors and the public using the company's website ([www.orchard-tx.com](http://www.orchard-tx.com)), the investor relations website ([ir.orchard-tx.com](http://ir.orchard-tx.com)), and on social media (Twitter and LinkedIn), including but not limited to investor presentations and investor fact sheets, U.S. Securities and Exchange Commission (SEC) filings, press releases, public conference calls and webcasts. The information that Orchard posts on these channels and websites could be deemed to be material information. As a result, Orchard encourages investors, the media, and others interested in Orchard to review the information that is posted on these channels, including the investor relations website, on a regular basis. This list of channels may be updated from time to time on Orchard's investor relations website and may include additional social media channels. The contents of Orchard's website or these channels, or any other website that may be accessed from its website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933.

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outcomes. All forward-looking statements by their nature address matters that involve risks and uncertainties, many of which are beyond Orchard's or Kyowa Kirin's control, and are not guarantees of future results, such as statements about the consummation of the proposed transaction and the anticipated benefits thereof. These and other forward-looking statements, are not guarantees of future results and are inherently subject to risks, uncertainties and assumptions that could cause actual results to differ materially from those expressed in any forward-looking statements. Accordingly, there are or will be important factors that could cause actual results to differ materially from those indicated in such statements and, therefore, you should not place undue reliance on any such statements and caution must be exercised in relying on forward-looking statements. Important risk factors and uncertainties that may cause such a difference include, but are not limited to, risks and uncertainties surrounding: (i) the completion of the proposed transaction on anticipated terms and timing, including in connection with obtaining shareholder and regulatory approvals, the sanction of the High Court of Justice of England and Wales, satisfaction of other closing conditions to consummate the acquisition, anticipated tax treatment, unforeseen liabilities, future capital expenditures, revenues, expenses, earnings, synergies, economic performance, indebtedness, financial condition, losses, future prospects, business and management strategies for the management, expansion and growth of Orchard's business and other conditions to the completion of the transaction; (ii) the occurrence of any event, change or other circumstance that could give rise to the termination of the definitive transaction agreement relating to the proposed transaction; (iii) Orchard's ability to implement its business model and strategic plans for its product, product candidates and pipeline, and challenges inherent in developing, commercializing, manufacturing, launching, marketing and selling existing and new products; (iv) significant transaction costs associated with the proposed transaction; (v) potential litigation relating to the proposed transaction; (vi) the risk that disruptions from the proposed transaction will harm Orchard's business, including current plans, operations and collaborations, and including as a result of diverting the attention of Orchard's and Kyowa Kirin's management from ongoing business operations; (vii) the ability of Orchard to retain and hire key personnel; (viii) potential adverse reactions or changes to business relationships resulting from the announcement or completion of the proposed transaction; (ix) legislative, regulatory and economic developments affecting Orchard's business; (x) general economic and market developments and conditions; (xi) the evolving legal, regulatory and tax regimes under which Orchard operates; (xii) potential business uncertainty, including changes to existing business relationships, during the pendency of the transaction that could affect Orchard's financial performance; (xiii) restrictions during the pendency of the proposed transaction that may impact Orchard's ability to pursue certain business opportunities or strategic transactions; (xiv) the risk that Orchard may be unable to obtain governmental and regulatory approvals required for the proposed transaction, or that required governmental and regulatory approvals may delay the consummation of the proposed transaction or result in the imposition of conditions that could reduce the anticipated benefits from the proposed transaction or cause the parties to abandon the proposed transaction; (xv) unpredictability and severity of catastrophic events, including, but not limited to, global pandemic, acts of terrorism or outbreak of war or hostilities, as well as Orchard's response to any of the aforementioned factors; (xvi) potential delays or failures related to research, clinical trials and/or development of Orchard's programs or product candidates, which are based on novel gene therapy and (xvii) the risks related to non-achievement of the CVR milestone and that holders of the CVRs will not receive payments in respect of the CVRs. Additional factors that may affect the future results of Orchard are set forth in Orchard's filings with the SEC, including Orchard's most recently filed Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the SEC, which are available on the SEC's website at [www.sec.gov](http://www.sec.gov). See in particular Item 1A of Orchard's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 and Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2023 under the headings "Risk Factors." The risks and uncertainties described above and in the SEC filings cited above are not exclusive and further information concerning Orchard and its business, including factors that potentially could materially affect Orchard's business, financial conditions or operating results, may emerge from time to time. Moreover, other risks and uncertainties of which Orchard is not currently aware may also affect Orchard's forward-looking statements and may cause actual results and the timing of events to differ materially from those anticipated. Readers are urged to consider these factors carefully in evaluating these forward-looking statements, and not to place undue reliance on any forward-looking statements, which speak only as of the date hereof and reflect the views stated therein with respect to future events as at such dates, even if they are subsequently made available by Orchard on its website or otherwise. Readers should also carefully review the risk factors described in other documents that Orchard files from time to time with the SEC. Except as required by law, Orchard assumes no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

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**No Offer or Solicitation**

This communication is not intended to and shall not constitute an offer to buy or sell or the solicitation of an offer to buy or sell any securities, or a solicitation of any vote or approval, nor shall there be any offer, solicitation or sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offer of securities shall be made in the United States absent registration under the U.S. Securities Act of 1933, as amended, or pursuant to an exemption from, or in a transaction not subject to, such registration requirements.

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**Condensed Consolidated Statements of Operations Data**  
(In thousands, except share and per share data)  
(Unaudited)

	Three Months Ended September 30,	
	2023	2022
Product revenue, net	\$ 5,559	\$ 5,377
Collaboration revenue	743	400
<b>Total revenue</b>	<b>6,302</b>	<b>5,777</b>
Costs and operating expenses:		
Cost of product revenue	1,745	1,645
Research and development	14,553	18,103
Selling, general and administrative	11,609	11,496
<b>Total costs and operating expenses</b>	<b>27,907</b>	<b>31,244</b>
<b>Loss from operations</b>	<b>(21,605)</b>	<b>(25,467)</b>
Other income (expense):		
Interest income	1,505	404
Interest expense	(932)	(799)
Changes in fair value of PIPE warrant and PIPE unit liabilities	(8,801)	—
Other income (expense), net	(4,510)	(22,787)
<b>Total other income (expense), net</b>	<b>(12,738)</b>	<b>(23,182)</b>
<b>Net loss before income taxes</b>	<b>(34,343)</b>	<b>(48,649)</b>
<b>Income tax benefit (expense)</b>	<b>(976)</b>	<b>1,084</b>
<b>Net loss attributable to ordinary shareholders</b>	<b>\$ (35,319)</b>	<b>\$ (47,565)</b>
<b>Net loss per ordinary share, basic and diluted</b>	<b>\$ (0.15)</b>	<b>\$ (0.37)</b>
<b>Weighted average ordinary shares outstanding, basic and diluted</b>	<b>228,388,561</b>	<b>128,132,092</b>

**Condensed Consolidated Balance Sheet Data**  
(In thousands)  
(Unaudited)

	September 30, 2023	December 31, 2022
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 23,469	\$ 68,424
Marketable securities	101,944	75,326
Accounts receivable	6,187	8,467
Inventory	6,638	3,400
Prepaid expenses and other current assets	7,451	6,586
Research and development tax credit receivable	8,234	5,942
Total current assets	<u>153,923</u>	<u>168,145</u>
Non-current assets:		
Operating lease right-of-use-assets	20,001	22,774
Property and equipment, net	7,131	8,138
Research and development tax credit receivable, net of current portion	3,372	—
Restricted cash	4,215	4,215
Intangible assets, net	3,321	3,560
Other assets	10,303	12,075
Total non-current assets	<u>48,343</u>	<u>50,762</u>
Total assets	<u>\$ 202,266</u>	<u>\$ 218,907</u>
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 3,661	\$ 9,318
Accrued expenses and other current liabilities	29,995	34,437
Deferred revenue	742	959
Operating lease liabilities	5,548	6,424
Notes payable	9,429	9,429
Total current liabilities	<u>49,375</u>	<u>60,567</u>
Notes payable, long-term	16,162	22,991
Deferred revenue, net of current portion	10,345	10,315
Operating lease liabilities, net of current portion	16,681	19,246
PIPE warrant liabilities	21,068	—
Other long-term liabilities	8,377	7,524
Total liabilities	<u>122,008</u>	<u>120,643</u>
Total shareholders' equity	<u>80,258</u>	<u>98,264</u>
Total liabilities and shareholders' equity	<u>\$ 202,266</u>	<u>\$ 218,907</u>

**Contact**

Benjamin Navon  
+1 857-248-9454  
Benjamin.Navon@orchard-tx.com

