



Orchard Therapeutics Reports Third Quarter 2022 Financial Results and Reviews Recent Business Highlights

November 14, 2022

Libmeldy® product sales totaled \$4.8M in Q3 and \$13.0M YTD with market expansion activities continuing in Europe

First case of MLD confirmed from newborn screening study provides critical evidence to advance adoption of universal screening in key countries

Clinical Type B meeting with U.S. FDA to take place in early 2023 prior to OTL-200 BLA submission

First neurocognitive data from OTL-201 POC trial in MPS-IIIa selected for oral presentation at 64th American Society of Hematology Annual Meeting & Exposition

Ended Q3 with \$146.6M of cash and investments and runway now into the second quarter of 2024

BOSTON and LONDON, Nov. 14, 2022 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced recent business highlights along with its financial results for the quarter ended September 30, 2022.

"Our accomplishments during the third quarter continue to distinguish Orchard as a commercial and scientific leader in HSC gene therapy," said Bobby Gaspar, M.D., Ph.D., chief executive officer. "Libmeldy's commercial traction in Europe continues to grow, and we are particularly encouraged by increasing disease awareness for MLD, as well as the positive screen from the ARCHIMEDlife pilot study, an important step toward the adoption of universal newborn screening. Heading into the end of the year, we continue to be in active dialogue with the FDA on our planned BLA submission for OTL-200 and look forward to sharing early cognitive outcomes from the ongoing OTL-201 proof-of-concept trial in MPS-IIIa as part of an oral presentation at the upcoming ASH meeting in December."

Frank Thomas, president and chief operating officer added, "Given our ongoing progress, I'm confident in Orchard's ability to become a breakout leader in the field of HSC gene therapy and am excited to be staying with the company in my current role. We've now extended our runway into the second quarter of 2024 due to savings realized from our restructuring earlier this year. This ensures we're appropriately resourced through the achievement of several significant near-term catalysts, including the potential U.S. approval of OTL-200."

Libmeldy Commercial Activities

- Orchard announced that the first confirmed case of a patient with metachromatic leukodystrophy (MLD) has been identified from the ARCHIMEDlife newborn screening (NBS) pilot study in collaboration with Hannover Screening Laboratory. The confirmation of a positive case supplies critical evidence necessary to advance the potential adoption of NBS in key markets. In total, over a dozen newborn screening studies have initiated in Europe, the Middle East and the U.S., with six studies actively screening newborns.
- Orchard has secured the renewal of the French early access program for Libmeldy, during which time the company would receive revenue for any eligible patient treated within the country.
- The company is also undergoing work to expand its treatment delivery network by qualifying centers to administer Libmeldy in Sweden and Spain, in addition to existing centers throughout Europe.

Data Publications

- Earlier this month, Orchard [announced](#) that clinical data, including early clinical outcomes of cognitive function, from the proof-of-concept (POC) trial of OTL-201 for MPS-IIIa have been accepted for an oral presentation at the 64th ASH Annual Meeting and Exposition, scheduled to take place December 10-13, 2022 in New Orleans, Louisiana.
- Ten presentations from Orchard's HSC platform were featured at the 29th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT), which took place October 11-14, 2022, in Edinburgh. Clinical and pre-clinical data from across the company's HSC gene therapy portfolio were featured in four oral and six poster presentations, including an invited talk from Bobby Gaspar, M.D., Ph.D., exploring the curative potential of HSC gene therapy when coupled with widespread newborn screening. Additional information on the presentations can be found [here](#).

Key Upcoming Corporate Milestones

Orchard is executing against the following list of upcoming expected milestones:

- **OTL-200 for MLD (U.S.):** Prior to scheduling a pre-BLA meeting, the FDA has requested completion of ongoing CMC interactions, and has recommended that Orchard request a Type B meeting to discuss specific elements of the clinical package. The company will provide an update following the clinical Type B meeting, which is anticipated for early 2023.
- **OTL-203 for MPS-IH:** Initiate a global registrational study in 2023.
- **OTL-201 for MPS-IIIa:** Report clinical data, including early clinical outcomes of cognitive function, from the proof-of-concept (POC) trial at the 2022 ASH Annual Meeting.
- **Research programs:**
 - Report preclinical POC data for the OTL-104 program in NOD2 Crohn's disease in early 2023 in advance of IND-enabling studies and an IND submission in 2024.
 - Continue to advance the OTL-105 program in hereditary angioedema (HAE), OTL-204 program for GRN-FTD and work in HSC-generated antigen-specific Tregs.

Third Quarter 2022 Financial Results

Revenue from product sales was \$5.4 million for the three months ended September 30, 2022, which included \$4.8 million in product sales of Libmeldy and \$0.6 million in product sales of Strimvelis. For the nine months ended September 30, 2022, revenue from product sales of Libmeldy totaled \$13.0 million. The cost of product sales was \$1.6 million during the third quarter of 2022 and includes the cost of manufacturing, royalties to third parties and non-cash amortization for both products.

Collaboration revenue was \$0.4 million for the three months ended September 30, 2022, resulting from the collaboration of OTL-105 in HAE with Pharming Group N.V. entered into in July 2021. This revenue represents reimbursements for research costs and preclinical studies incurred by the company and a portion of the \$17.5 million upfront consideration received by Orchard under the collaboration, which is being amortized into revenue over the expected duration of the agreement.

Research and development expenses were \$18.1 million for the three months ended September 30, 2022, compared to \$20.8 million in the same period in 2021. R&D expenses decreased 13% for the period compared to the prior year and declined 36% versus the first quarter of 2022, following the Company's corporate restructuring. R&D expenses include the costs of clinical trials and preclinical work on the company's portfolio of investigational gene therapies, as well as costs related to regulatory, manufacturing, license fees and milestone payments under the company's agreements with third parties, and personnel costs to support these activities.

Selling, general and administrative expenses were \$11.5 million for the three months ended June 30, 2022, compared to \$13.0 million in the same period in 2021, a decrease of 12%. The decline resulted primarily from the realization of savings from the restructuring announced in March 2022, partially offset by increasing commercialization expenses to support a potential U.S. launch of Libmeldy in 2023.

The Company expects to continue to realize additional benefits from the March 2022 restructuring and expects any savings to be fully reflected in its operating expenses by year end 2022.

The loss from operations was \$25.5 million in the third quarter of 2022 compared to a loss from operations of \$32.9 million in the corresponding period of 2021. The reduction resulted from both increased revenue from product sales as well as reduced operating expenses following the March 2022 corporate restructuring.

Since the beginning of 2022, the U.S. dollar has strengthened against the British pound and Euro, resulting in unrealized losses on certain intercompany balances denominated in currencies other than the U.S. dollar. During the quarter, the company had unrealized losses on foreign currency balances of approximately \$21.5 million that are subject to future exchange rate fluctuations. Most of the Company's cash balance has been and continues to be held in U.S. dollars.

Net loss was \$47.6 million for the three months ended September 30, 2022, compared to \$36.4 million in the same period in 2021. The company had approximately 128.1 million ordinary shares outstanding as of September 30, 2022.

Cash, cash equivalents and investments as of September 30, 2022, were \$146.6 million, with \$33.0 million of debt outstanding, compared to \$220.1 million and the same debt figure as of December 31, 2021. The Company expects that its existing cash, cash equivalents and investments will now fund its anticipated operating, debt service and capital expenditure requirements into the second quarter of 2024.

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 bone marrow harvest or 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 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, and follow us on [Twitter](#) and [LinkedIn](#).

Availability of Other Information About Orchard Therapeutics

Investors and others should note that Orchard communicates with its investors and the public using the company website (www.orchard-tx.com), the investor relations website (ir.orchard-tx.com), and on social media (twitter.com/orchard_tx and www.linkedin.com/company/orchard-therapeutics), including but not limited to investor presentations and investor fact sheets, U.S. Securities and Exchange Commission 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.

Forward-looking Statements

This press release contains certain forward-looking statements about Orchard's strategy, future plans and prospects, which are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements may be identified by words such as "anticipates," "believes," and "expects," or similar expressions, which are intended to identify forward-looking statements. Forward-looking statements include express or implied statements relating to, among other things, Orchard's business strategy and goals, the therapeutic potential of Orchard's products and product candidates, including the products and product candidates referred to in this release, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, including the product candidates referred to in this release, the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates, including the anticipated timing of a Type B meeting with the FDA regarding OTL-200, the timing of a potential registrational study for OTL-203 for MPS-IH, the timing of announcement of clinical data for its product candidates, including the anticipated timing of clinical data for OTL-201 and preclinical data for OTL-104, the likelihood that such data will be positive and support further clinical development and regulatory approval of these product candidates, the likelihood of approval of such product candidates by the applicable regulatory authorities, the expected savings to Orchard's business as a result of the organizational updates referred to in this release, and the company's financial condition and cash runway into 2024. These statements are neither promises nor guarantees and are subject to a variety of risks and uncertainties, many of which are beyond Orchard's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. In particular, these risks and uncertainties include, without limitation: the risk that Orchard will not realize the anticipated benefits of its strategic plan or the expected cash savings from its restructuring; the risk that any one or more of Orchard's product candidates, including the product candidates referred to in this release, will not be approved, successfully developed or commercialized; the risk of cessation or delay of any of Orchard's ongoing or planned clinical trials; the risk that Orchard may not successfully recruit or enroll a sufficient number of patients for its clinical trials; the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving Orchard's product candidates; the delay of any of Orchard's regulatory submissions; the failure to obtain or maintain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates or the receipt of restricted marketing approvals; the risk of delays in Orchard's ability to commercialize its product candidates, if approved; the risk that the ongoing and evolving COVID-19 pandemic, or global macroeconomic and geopolitical developments, could affect the company's business; and the risk that the market opportunity for Libmeldy and its product candidates may be lower than estimated or that Orchard may be unable to identify patients for its products on a consistent basis. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements.

Other risks and uncertainties faced by Orchard include those identified under the heading "Risk Factors" in Orchard's most recent annual or quarterly report filed with the U.S. Securities and Exchange Commission (SEC), as well as subsequent filings and reports filed with the SEC. The forward-looking statements contained in this press release reflect Orchard's views as of the date hereof, and Orchard does not assume and specifically disclaims any obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|-------------------------------|----------------------------------|-------|---------------------------------|-------|
| | 2022 | 2021 | 2022 | 2021 |
| Revenue: | | | | |
| Libmeldy product sales | \$ 4,781 | \$ — | \$ 12,985 | \$ — |
| Strimvelis product sales | 596 | 700 | 1,232 | 700 |
| Collaboration revenue | 400 | 492 | 1,452 | 492 |
| Total revenue | 5,777 | 1,192 | 15,669 | 1,192 |
| Costs and operating expenses: | | | | |

| | | | | |
|--|--------------------|--------------------|---------------------|---------------------|
| Cost of product sales | 1,645 | 226 | 4,338 | 226 |
| Research and development | 18,103 | 20,846 | 68,302 | 63,631 |
| Selling, general and administrative | 11,496 | 13,039 | 38,525 | 41,353 |
| Total costs and operating expenses | <u>31,244</u> | <u>34,111</u> | <u>111,165</u> | <u>105,210</u> |
| Loss from operations | <u>(25,467)</u> | <u>(32,919)</u> | <u>(95,496)</u> | <u>(104,018)</u> |
| Other income (expense): | | | | |
| Interest income | 404 | 65 | 686 | 349 |
| Interest expense | (799) | (683) | (2,146) | (1,814) |
| Other income (expense), net | <u>(22,787)</u> | <u>(4,041)</u> | <u>(47,066)</u> | <u>(2,049)</u> |
| Total other income (expense), net | <u>(23,182)</u> | <u>(4,659)</u> | <u>(48,526)</u> | <u>(3,514)</u> |
| Loss before income taxes | <u>(48,649)</u> | <u>(37,578)</u> | <u>(144,022)</u> | <u>(107,532)</u> |
| Income tax (expense) benefit | <u>1,084</u> | <u>1,133</u> | <u>1,245</u> | <u>(704)</u> |
| Net loss | <u>\$ (47,565)</u> | <u>\$ (36,445)</u> | <u>\$ (142,777)</u> | <u>\$ (108,236)</u> |
| Net loss per share, basic and diluted | <u>\$ (0.37)</u> | <u>\$ (0.29)</u> | <u>\$ (1.12)</u> | <u>\$ (0.88)</u> |
| Weighted average number of shares outstanding, basic and diluted | <u>128,132,092</u> | <u>127,376,562</u> | <u>127,895,426</u> | <u>122,765,516</u> |

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

| | <u>September 30,</u> <u>2022</u> | <u>December 31,</u> <u>2021</u> |
|---|-------------------------------------|------------------------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 80,751 | \$ 55,912 |
| Marketable securities | 65,803 | 164,195 |
| Accounts receivable | 4,233 | 1,480 |
| Prepaid expenses and other current assets | 16,991 | 23,011 |
| Research and development tax credit receivable | <u>11,475</u> | <u>30,723</u> |
| Total current assets | 179,253 | 275,321 |
| Operating lease right-of-use-assets | 23,133 | 24,316 |
| Property and equipment, net | 7,804 | 4,767 |
| Restricted cash | 4,266 | 4,266 |
| Intangible assets, net | 3,487 | 4,149 |
| Research and development tax credit receivable | 4,312 | — |
| Other assets | <u>11,795</u> | <u>9,590</u> |
| Total assets | <u>\$ 234,050</u> | <u>\$ 322,409</u> |
| Liabilities and Shareholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 6,518 | \$ 10,008 |
| Accrued expenses and other current liabilities | 28,267 | 24,318 |
| Deferred revenue, current | 1,375 | 346 |
| Operating lease liabilities | 6,248 | 7,335 |
| Notes payable, current | <u>7,857</u> | <u>786</u> |
| Total current liabilities | 50,265 | 42,793 |
| Notes payable, long-term | 25,265 | 32,086 |
| Deferred revenue, net of current portion | 9,101 | 12,519 |
| Operating lease liabilities, net of current portion | 19,083 | 19,278 |
| Other long-term liabilities | <u>6,633</u> | <u>5,783</u> |
| Total liabilities | <u>110,347</u> | <u>112,459</u> |
| Total shareholders' equity | <u>123,703</u> | <u>209,950</u> |
| Total liabilities and shareholders' equity | <u>\$ 234,050</u> | <u>\$ 322,409</u> |