Orchard Therapeutics Reviews Recent Portfolio Progress and Reports Third Quarter 2020 Financial Results

November 3, 2020

Libmeldy [TM] Receives Positive CHMP Opinion for the Treatment of Early-Onset Metachromatic Leukodystrophy (MLD); U.S. IND Application on Track for Year End 2020

Cash and Investments of More Than $200M and Runway into 2022

Frontotemporal dementia (FTD) and Crohn's Disease Preclinical Programs to be Featured in Virtual R&D Event on November 13, 2020

BOSTON and LONDON, Nov. 03, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today reviewed recent business accomplishments and reported financial results for the quarter ended September 30, 2020.

"The positive CHMP opinion we received last month for Libmeldy in the EU represents a proud moment for Orchard and offers a potentially transformative therapy for early-onset MLD patients and their families," said Bobby Gaspar, M.D., Ph.D., chief executive officer, Orchard Therapeutics. "We are looking forward to many exciting developments for this program and the rest of our pipeline in the coming months, including our November investor event which will highlight our work in conditions with larger patient populations such as FTD and Crohn's disease."

Recent Corporate Achievements

- **Libmeldy (OTL-200) for MLD:**
  - Received a positive CHMP opinion recommending full marketing authorization for the treatment of early-onset metachromatic leukodystrophy (MLD) patients in the European Union (EU). The CHMP's positive opinion will be reviewed by the European Commission (EC), and a final decision is anticipated before the end of 2020. If approved, Libmeldy would be the first commercial therapy and first *ex vivo* hematopoietic stem cell (HSC) lentiviral gene therapy for eligible patients with early-onset MLD. Link to full release here.
- **OTL-203 for MPS-I:**
  - Received EMA PRIME designation for the treatment of MPS-I. Link to full release here.
  - Presented additional interim data from the ongoing proof-of-concept (POC) trial of OTL-203 at the 46th Annual Meeting of the European Society for Blood and Bone Marrow Transplantation (EBMT).The data demonstrate sustained hematologic engraftment for all eight patients treated with preliminary clinical data showing improved motor function, stable cognitive scores and continued normal growth from the first two patients treated with at least one year of follow-up. Link to full release here.

Planned Corporate Milestones

Libmeldy (OTL-200) for MLD	Obtain EC approval by year-end 2020; launch in the EU in 1H 2021; Seek regenerative medicine advanced therapy (RMAT) designation and file an investigational new drug (IND) application in U.S. by year-end 2020
OTL-103 for WAS	Submit U.S. biologics license application (BLA) and EU marketing authorization application (MAA) filings in 2021
OTL-203 for MPS-I	Report one-year follow-up results and initiate a registrational study in 2021
OTL-201 for MPS-IIIA	Complete enrollment in POC study and release interim data in 2021
Research	Provide detail on pre-clinical development in FTD and Crohn's disease programs at November R&D event

Third Quarter 2020 Financial Results

Revenue from product sales of Strimvelis were $2.0 million for the third quarter of 2020 compared to $1.9 million in the same period in 2019, and cost of product sales were $0.7 million for the third quarter of 2020 compared to $0.6 million in the same period in 2019.

Research and development (R&D) expenses were $14.7 million for the third quarter of 2020 compared to $28.5 million in the same period in 2019. 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. The decline in R&D expenses is primarily attributable to the company's U.K. research and development tax credits, which are recorded as an offset to R&D expense. During the third quarter of 2020, the company recorded tax credits of $10.1 million as compared to $2.7 million

in the same period in 2019. Further, the company has realized savings associated with an updated strategy and corporate restructuring previously announced in May 2020, including the consolidation of its R&D sites.

Selling, general and administrative expenses were $13.0 million for the third quarter of 2020 compared to $14.2 million in the same period in 2019. The decline was primarily due to lower administrative and corporate expenses as compared to the prior period, primarily resulting from the May 2020 corporate restructuring.

Net loss was $20.3 million for the third quarter of 2020 compared to $36.7 million in the same period in 2019. The lower net loss compared to the same period in 2019 is a result of the higher U.K. research and development tax credits, the savings associated with an updated strategic plan and corporate restructuring, and $5.5 million in other income primarily attributable to unrealized foreign currency gains in the quarter. The company had 97.7 million ordinary shares outstanding as of September 30, 2020.

Cash, cash equivalents and investments as of September 30, 2020, were $201.3 million compared to $325.0 million as of December 31, 2019. The decrease was primarily driven by cash used to fund operations for the nine months ended September 30, 2020. In the third quarter of 2020, the company received approximately $13.6 million of cash from R&D tax credits related to 2018 as a result of qualifying activities under the tax code in the U.K. The company expects that its existing cash, cash equivalents and investments will fund its anticipated operating and capital expenditure requirements into 2022. This excludes the $50 million expected to be available under the company's credit facility and any non-dilutive capital received from potential future partnerships or priority review vouchers.

"Our European commercial team is in place and have executed the activities necessary to launch Libmeldy (if approved) on a country-by-country basis in the first half of 2021," said Frank Thomas, president and chief operating officer. "In addition, we will be leveraging cross border reimbursement channels for the treatment of patients from other parts of the world based on our past experience with Strimvelis."

Webcast Information for November 13 R&D Investor Event

The company will be webcasting a virtual R&D investor event starting at 9:00 am ET on Friday, November 13, 2020. The event can be accessed under "News & Events" in the "Investors & Media" section of the company's website at www.orchard-tx.com, and a replay will be archived on the Orchard website following the event.

About Orchard

Orchard Therapeutics is a global gene therapy leader dedicated to transforming the lives of people affected by rare diseases through the development of innovative, potentially curative gene therapies. Our *ex vivo* autologous gene therapy approach harnesses the power of genetically modified blood stem cells and seeks to correct the underlying cause of disease in a single administration. In 2018, Orchard 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. Orchard now has one of the deepest and most advanced gene therapy product candidate pipelines in the industry spanning multiple therapeutic areas where the disease burden on children, families and caregivers is immense 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

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 and LinkedIn), 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," "expects," "plans," "intends," "projects," and "future" or similar expressions that 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, including its plans and expectations for the regulatory approval and commercialization of Libmeldy, the therapeutic potential of Orchard's product candidates, including Libmeldy and the other 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, the timing of announcement of clinical data for its product candidates, 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, and Orchard's financial condition and cash runway into 2022. 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 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, including Libmeldy, will be insufficient to support regulatory submissions or marketing approval in the US and EU or that long-term adverse safety findings may be discovered; the delay of any of Orchard's regulatory submissions; the failure to obtain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates, including the risk that our marketing authorization application submitted for Libmeldy may not be approved by the European Commission when expected, or at all, or the receipt of restricted marketing approvals; the inability or risk of delays in Orchard's ability to commercialize its product

candidates, including Libmeldy, if approved, including the risk that Orchard may not secure adequate pricing or reimbursement to support continued development or commercialization of Libmeldy; the risk that the market opportunity for its product candidates, including Libmeldy, may be lower than estimated; and the severity of the impact of the COVID-19 pandemic on Orchard's business, including on clinical development, its supply chain and commercial programs. 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 quarterly report on Form 10-Q for the quarter ended September 30, 2020, to be 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
(In thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,	
	2020	**2019**
Product sales, net	$ 1,998	$ 1,918
Costs and operating expenses:		
Cost of product sales	667	614
Research and development	14,678	28,493
Selling, general and administrative	12,956	14,223
Total costs and operating expenses	28,301	43,330
Loss from operations	(26,303)	(41,412)
Other income (expense):		
Interest income	534	2,169
Interest expense	(572)	(660)
Other income (expense), net	5,510	972
Total other income (expense), net	5,472	2,481
Net loss before income tax	(20,831)	(38,931)
Income tax (expense) benefit	541	2,194
Net loss attributable to ordinary shareholders	(20,290)	(36,737)
Net loss per share attributable to ordinary shareholders, basic and diluted	$ (0.20)	$ (0.38)
Weighted average number of ordinary shares outstanding, basic and diluted	99,664,616	97,817,847

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

	September 30, 2020	**December 31, 2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 41,161	$ 19,053
Marketable securities	160,183	305,937
Trade receivables	3,183	1,442
Prepaid expenses and other current assets	11,537	8,530
Research and development tax credit receivable, current	18,047	14,934
Total current assets	234,111	349,896
Non-current assets:		
Operating lease right-of-use-assets	30,476	19,415
Property and equipment, net	4,912	7,596
Research and development tax credit receivable	12,440	13,710
Other assets	16,376	8,664

Total assets	$ 298,315	$ 399,281
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 5,861	$ 11,984
Accrued expenses and other current liabilities	23,135	37,980
Operating lease liabilities	8,990	5,892
Notes payable, current	2,778	—
Total current liabilities	40,764	55,856
Notes payable, long-term	22,199	24,699
Operating lease liabilities, net of current portion	24,162	15,320
Other long-term liabilities	4,123	4,213
Total liabilities	91,248	100,088
Shareholders' equity	207,067	299,193
Total liabilities and shareholders' equity	$ 298,315	$ 399,281

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