Orchard Therapeutics Reports 2019 Financial Results and Reviews Key Strategic Priorities for 2020

February 27, 2020

Commercial Preparations on Track for Potential Second Half 2020 EU Launch of OTL-200 for MLD with U.S. Regulatory Filing Expected Late 2020 / Early 2021

Initiation of Rolling U.S. Regulatory Filing for OTL-101 (ADA-SCID) Planned for First Half 2020

Recent WORLD Presentations Showcased Data from Neurometabolic Franchise; Additional Clinical Data from MPS-I and MPS-IIIA Proof of Concept Trials Expected in 2020

$325M in Cash and Investments to Support Execution on Strategic Priorities in 2020 and Beyond

BOSTON and LONDON, Feb. 27, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global leader in gene therapy, today reported business highlights and financial results for the year ended December 31, 2019, as well as 2020 strategic priorities and upcoming milestones.

"We are inspired by the possibilities ahead for Orchard in 2020 and beyond to bring the benefits of our gene therapy approach to patients worldwide," said Mark Rothera, president and chief executive officer of Orchard. "As we prepare for the anticipated EU approval of OTL-200 for MLD, we are strengthening the foundation of our global commercial infrastructure that could one day support multiple potentially transformative products. We are also continuing to propel the business forward by advancing our next wave of proof-of-concept trials evaluating the potential for gene-corrected stem cells in a broader range of neurometabolic disorders. With strong execution in 2019 and a solid balance sheet heading into 2020, we are well-positioned to deliver value to our shareholders."

Recent 2020 Achievements

- Presented data from the company's neurometabolic franchise at the 16th Annual WORLD *Symposium* in Orlando, Florida including:
  - **MLD:**
    - Data from a total of 33 patients treated with OTL-200, a gene therapy under an accelerated assessment review by the European Medicines Agency (EMA) for the treatment of metachromatic leukodystrophy (MLD).
    - Emerging data from a separate study quantifying caregiver-reported quality of life experiences was shown and describes the immense burden on their financial, professional, psychosocial and physical well-being.
  - **MPS-I:** Data from the ongoing proof-of-concept trial of OTL-203 for the treatment of mucopolysaccharidosis type I (MPS-I). Evidence of engraftment and overexpression of the alpha-l-iduronidase (IDUA) enzyme was seen in all evaluable patients, with a median follow-up of six months (range of 1 - 12 months).
  - **MPS-IIIA:** A case report of the first patient treated with *ex-vivo,* autologous hematopoietic stem cell (HSC) gene therapy for mucopolysaccharidosis type IIIA (MPS-IIIA) under compassionate use was presented by Royal Manchester Children's Hospital [1].
- Appointed industry veteran Steven Altschuler, M.D. to its board of directors. Dr. Altschuler brings nearly 20 years of experience in the healthcare industry and currently serves as managing director of Healthcare Ventures at Ziff Capital Partners. He was previously chair of the board of directors at Spark Therapeutics (now part of Roche), a commercial-stage gene therapy company.
- Announced that the FDA has granted orphan drug designation for OTL-102 for the treatment of X-linked chronic granulomatous disease (X-CGD).

2020 / 2021 Corporate Priorities and Expected Key Milestones

- Obtain approval for and launch OTL-200 for the treatment of MLD in Europe in the second half of 2020.
- Submit a biologics license application (BLA) filing in the U.S. for OTL-200 for the treatment of MLD in late 2020 or early 2021.
- Initiate a rolling BLA filing in the U.S. for OTL-101 in adenosine deaminase severe combined immunodeficiency (ADA-SCID) in the first half of 2020 with anticipated completion of the filing within 12 months of initiation.
- Prepare for BLA and marketing authorization application (MAA) regulatory filings for OTL-103 in Wiskott-Aldrich Syndrome (WAS) in the U.S. and EU in 2021.
- Release interim data from the OTL-203 proof-of-concept clinical trial in MPS-I in 2020. One-year follow-up results for the first eight patients, including the primary endpoints, are anticipated in 2021.
- Enroll five patients and release interim data on the first patient from the recently initiated proof-of-concept clinical trial for OTL-201 in MPS-IIIA in 2020.

- Initiate construction of an in-house manufacturing facility in Fremont, California in 2020 (design of the facility is complete).

1 Patient was treated by the Royal Manchester Children's Hospital (RMCH) under a "Specials" license, granted by the UK government for the use of an unlicensed pharmaceutical product in situations of high unmet need when there is no other treatment option available. Orchard holds the license to the MPS-IIIA investigational gene therapy product (OTL-201) and is funding the proof-of-concept clinical trial being conducted at RMCH, which utilizes the same technology and procedures that were used to treat this first MPS-IIIA patient.

Fourth Quarter 2019 Financial Results

Cash, cash equivalents and investments as of December 31, 2019, were $325.0 million compared to $335.8 million as of December 31, 2018. The decrease was primarily driven by the net cash used to fund operations in 2019, partially offset by proceeds from the company's public equity offering in June 2019 and the proceeds from the first drawdown under a credit facility entered in May 2019. During the three months ended December 31, 2019, the company's cash used to fund operations and capital expenditures totaled approximately $43.8 million. The quarterly burn rate is expected to increase in 2020 due to the growth in operating expenses to support the potential launch of OTL-200 in the second half of 2020 and the company's planned capital investment on its in-house manufacturing facility.

During the three months ended December 31, 2019, the company recognized $0.6 million in revenue related to European sales of Strimvelis®, the first gene therapy approved by the EMA for ADA-SCID.

Research and development expenses were $30.9 million for the three months ended December 31, 2019, compared to $17.4 million in the same period in 2018. 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 company expects R&D expenses to continue to increase as its programs advance through development.

Selling, general and administrative expenses were $18.5 million for the three months ended December 31, 2019, compared to $12.0 million in the same period in 2018. The increase was primarily due to increased investment to prepare for the potential commercialization of multiple late-stage programs, as well as higher costs to support public company operations and stock-based compensation.

Net loss attributable to ordinary shareholders was $45.4 million for the three months ended December 31, 2019, compared to $25.1 million in the same period in 2018. The increase in net loss as compared to the prior year was primarily due to higher costs related to pre-launch activities on the company's later-stage programs in development and expenses associated with being a public company. The company had 96.9 million ordinary shares outstanding as of December 31, 2019.

The company expects that its existing cash, cash equivalents and investments will enable funding of its anticipated operating and capital expenditure requirements into the second half of 2021.

Conference Call & Webcast Information

Orchard will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss its 2019 financial results and other business updates. To participate in the conference call, please dial 866-930-5155 (U.S. domestic) or +1-409-937-8974 (international) and refer to conference ID 8096875. A live webcast of the presentation will be available under "News & Events" in the "Investors & Media" section of the company's website at orchard-tx.com and a replay will be archived on the Orchard website following the presentation.

About Orchard
Orchard Therapeutics is a global gene therapy leader dedicated to transforming the lives of people affected by rare diseases through innovative, potentially curative gene therapies. Our *ex vivo* autologous gene therapy approach harnesses the power of genetically-modified blood stem cells and seeks to permanently correct the underlying cause of disease in a single administration. The company has one of the deepest gene therapy pipelines in the industry and is advancing seven clinical-stage programs across multiple therapeutic areas where the disease burden on children, families and caregivers is immense and current treatment options are limited or do not exist, including inherited neurometabolic disorders, primary immune deficiencies and blood disorders.

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.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," "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, the company's business strategy and goals, the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, including the product candidate or 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 and 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 likelihood the company will initiate construction of an in-house manufacturing facility in 2020, and the company's financial condition and cash runway into the second half of 2021. 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, the risks and uncertainties include, without limitation: the risk that any one or more of Orchard's product candidates, including the product candidate or 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 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 marketing approval from the applicable regulatory authorities for any of Orchard's product candidates, the receipt of restricted marketing approvals, and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. 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 annual report on Form 20-F for the year ended December 31, 2018, as filed with the U.S. Securities and Exchange Commission (SEC) on March 22, 2019, 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.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Product sales, net	$ 595	$ 689	$ 2,513	$ 2,076
Costs and operating expenses:				
Cost of product sales	191	142	805	422
Research and development	30,899	17,426	117,363	205,319
Selling, general and administrative	18,531	11,952	57,218	31,366
Total costs and operating expenses	49,621	29,520	175,386	237,107
Loss from operations	(49,026)	(28,831)	(172,873)	(235,031)
Other income (expense):				
Interest income	1,843	1,114	7,362	1,116
Interest expense	(633)	—	(1,538)	—
Other income (expense), net	2,533	3,056	1,387	4,390
Total other income (expense), net	3,743	4,170	7,211	5,506
Net loss before income tax	(45,283)	(24,661)	(165,662)	(229,525)
Income tax (expense) benefit	(133)	(402)	2,240	(970)
Net loss attributable to ordinary shareholders	(45,416)	(25,063)	(163,422)	(230,495)
Net loss per share attributable to ordinary shareholders, basic and diluted	$ (0.46)	$ (0.42)	$ (1.75)	$ (10.22)
Weighted average number of ordinary shares outstanding, basic and diluted	98,243,915	59,435,325	93,240,355	22,559,389

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 19,053	$ 335,844
Marketable securities	305,937	—
Trade receivables	1,442	2,103

Prepaid expenses and other assets	8,530	6,985
Research and development tax credit receivable, current	14,934	10,585
Total current assets	$ 349,896	$ 355,517
Non-current assets:		
Operating lease right-of-use assets	19,415	—
Property and equipment, net	7,596	5,476
Research and development tax credit receivable	13,710	—
Other long-term assets	8,664	5,049
Total non-current assets	49,385	10,525
Total assets	$ 399,281	$ 366,042
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 11,984	$ 18,125
Accrued expenses and other current liabilities	37,980	29,780
Operating lease liabilities	5,892	—
Total current liabilities	55,856	47,905
Long-term debt, net	24,699	—
Operating lease liabilities, non-current	15,320	—
Other long-term liabilities	4,213	6,799
Total liabilities	100,088	54,704
Shareholders’ equity:	299,193	311,338
Total liabilities and shareholders’ equity	$ 399,281	$ 366,042

Contacts

Investors
Renee Leck
Director, Investor Relations
+1 862-242-0764
Renee.Leck@orchard-tx.com

Media
Molly Cameron
Manager, Corporate Communications
+1 978-339-3378
media@orchard-tx.com

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