Orchard Therapeutics Reports 2018 Financial Results and Reviews Recent Business Highlights

March 21, 2019

Three Lead Programs for MLD, ADA-SCID and WAS on Track for Regulatory Filings Over the Next Three Years, with MLD and ADA-SCID Expected in 2020

Registrational Data Showing 100% Overall Survival and 100% Event-Free Survival at Two Years in ADA-SCID Patients Recently Presented at ASBMT; Two and Three Year Data in MLD Patients to be Presented Next Week at EBMT

Recent CTO Appointment Enhances Global Commercial Manufacturing Capabilities

Strong Financial Position with Approximately $340M in Total Cash and Investments

BOSTON and LONDON, March 21, 2019 (GLOBE NEWSWIRE) -- Orchard Therapeutics (NASDAQ: ORTX), a leading commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies, today reported business highlights and financial results for the year ended December 31, 2018, as well as upcoming 2019 milestones.

"In 2018 we expanded and advanced our portfolio of autologous, *ex vivo*, hematopoietic stem cell gene therapies and established the capabilities to scale a global, fully integrated biopharmaceutical company serving patients. This year, we plan to build on these successes and further establish Orchard as a leader in the gene therapy field," said Mark Rothera, president and chief executive officer of Orchard. "Our priorities include advancing our ADA-SCID and MLD programs towards regulatory filings in 2020 and preparing the ground for their commercialization while extending our clinical stage pipeline. This work underpins our vision of delivering therapies that have the potential to transform the lives of patients with rare, life-threatening diseases around the globe with a single treatment."

Recent Highlights

- Presented two-year follow-up data in 20 patients from the fresh formulation registrational trial of OTL-101 in patients with severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID) at the 2019 Transplantation and Cellular Therapy Meetings of ASBMT and CIBMTR. Patients treated with OTL-101 in this trial showed 100% overall survival and 100% event free survival at 24 months, demonstrating favorable outcomes compared to a historical control group of patients who received a hematopoietic stem cell transplant (link to full data here).
- Presented clinical proof of concept data for OTL-102 for the treatment of X-linked chronic granulomatous disease (X-CGD), also at ASBMT. Six patients receiving OTL-102 continue to show sustained levels of functioning neutrophils 12 months after treatment with gene therapy and no longer receive CGD-related prophylactic antibiotics (link to full data here). This represents the company's fourth program to reach clinical proof of concept.
- Recognized the publication in *Nature Medicine* by the San Raffaele-Telethon Institute for Gene Therapy of OTL-300 interim clinical data in seven patients with transfusion-dependent beta-thalassemia (TDBT).
- Appointed Ran Zheng as chief technical officer. In this role Ms. Zheng will be responsible for global manufacturing and technical operations. Ms. Zheng spent the last 16 years in roles of increasing responsibility within operations at Amgen, most recently as a vice president overseeing end-to-end supply chain and manufacturing operations to enable global clinical development, product launch and commercial supply.

Upcoming Data Presentations & Remaining 2019 Milestones

Neurometabolic Disorders

- Present two and three-year follow-up data in 20 patients from the fresh formulation registrational trial of OTL-200 for metachromatic leukodystrophy (MLD) at the 45th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT) on March 26 in Frankfurt, Germany
- Release engraftment data in the first three patients from the cryopreserved formulation clinical trial of OTL-200 for MLD
- Support the submission of a clinical trial application for OTL-201 for mucopolysaccharidosis type IIIA (MPS-IIIA) in preparation of a clinical trial initiation

Primary Immune Deficiencies

- Release engraftment data in 10 patients from a cryopreserved formulation clinical trial of OTL-101 for ADA-SCID
- Publish three-year follow-up data in eight patients from the fresh formulation registrational trial of OTL-103 for Wiskott-Aldrich syndrome (WAS)
- Initiate enrollment in a cryopreservation formulation clinical trial for OTL-103 for patients with WAS
- Design and engage regulators on the registrational trial for OTL-102 for patients with X-CGD

Hemoglobinopathies

- Report clinical proof-of-concept data in nine patients at 12 months for OTL-300 in TDBT

Full Year 2018 Financial Results

Cash and investments as of December 31, 2018 were $339.7 million, including $3.8 million of restricted cash, compared to $89.9 million as of December 31, 2017. The increase was primarily driven by proceeds from the Series C financing in August 2018 and initial public offering in November 2018, offset by cash used to fund operations in 2018.

During 2018, the company recognized $2.1 million in net product sales from Strimvelis. Strimvelis is approved in Europe to treat ADA-SCID and was acquired from GSK in April 2018.

Research and development expenses were $205.3 million in 2018, compared to $32.5 million in 2017. The increase was primarily driven by the acquisition of Strimvelis and three clinical-stage programs acquired from GSK in April 2018. As a result of this transaction, the company recognized a one-time charge to research and development expenses of $133.6 million related to in-process research and development programs that had no future alternative use at the closing.

Selling, general and administrative expenses were $31.4 million in 2018, compared to $6.0 million in 2017. The increase was primarily due to personnel costs to support public company operations, as well as costs to market Strimvelis and prepare for the potential commercialization of the company's late-stage development programs.

Net loss attributable to ordinary shareholders was $230.5 million in 2018, compared to $39.7 million in 2017. The increase resulted primarily from the one-time charge related to the GSK transaction.

The company expects that its cash and investments as of December 31, 2018 will fund its anticipated operating and capital expenditure requirements into the second half of 2020.

Conference Call & Webcast Information

Orchard will host a conference call and live webcast with slides today at 8:00 a.m. ET to discuss full year 2018 financial results and recent business activities. To participate in the conference call, please dial 1-866-930-5155 (domestic) or 1-409-937-8974 (international) and referring to conference ID 2279788. 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. A replay of the webcast will be archived on the Orchard website following the presentation.

About Orchard

Orchard Therapeutics is a fully integrated commercial-stage biopharmaceutical company dedicated to transforming the lives of patients with serious and life-threatening rare diseases through innovative gene therapies.

Orchard's portfolio of autologous, ex vivo, hematopoietic stem cell gene therapies includes Strimvelis, the first such treatment approved by the European Medicines Agency for severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID). Additional programs for neurometabolic disorders, primary immune deficiencies and hemoglobinopathies include three advanced registrational studies for metachromatic leukodystrophy (MLD), ADA-SCID and Wiskott-Aldrich syndrome (WAS), clinical programs for X-linked chronic granulomatous disease (X-CGD) and transfusion-dependent beta-thalassemia (TDBT), as well as an extensive preclinical pipeline. The programs in MLD, WAS and TDBT Orchard acquired from GSK in April 2018 and originated from a pioneering collaboration between GSK and the San Raffaele Telethon Institute for Gene Therapy (Milan, Italy) initiated in 2010.

Orchard currently has offices in the U.K. and the U.S., including London, San Francisco and Boston.

Forward-Looking Statements
This press release contains certain forward-looking statements 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,” “intends,” “projects,” “anticipates,” 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 expectations regarding the timing of regulatory submissions for approval of its product candidates, 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, and the Company's financial condition and cash runway through the second half of 2020. These statements are neither promises nor guarantees but 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 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, or 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. For additional disclosure regarding these and other risks faced by Orchard, see the disclosure contained in Orchard's public filings with the Securities and Exchange Commission, including in the final prospectus related to Orchard's initial public offering filed with the Securities and Exchange Commission pursuant to Rule 424(b) of the Securities Act of 1933, as amended, as well as subsequent filings and reports filed by Orchard with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of publication of this document. Orchard undertakes no 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)

	Six Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
Product sales, net	$ 2,076	$ —	$ 2,076	$ —
Costs and operating expenses				
Cost of product sales	422	—	422	—
Research and development	45,157	21,981	205,319	32,527
Selling, general and administrative	19,418	3,715	31,366	5,985
Total costs and operating expenses	64,997	25,696	237,107	38,512
Loss from operations	(62,921)	(25,696)	(235,031)	(38,512)
Other income (expense):				
Total other income (expense), net	5,105	(779)	5,506	(1,179)
Net loss before income tax	(57,816)	(26,475)	(229,525)	(39,691)
Income tax expense	(1,135)	(95)	(970)	(53)
Net loss attributable to ordinary shareholders	$ (58,951)	$ (26,570)	$ (230,495)	$ (39,744)
Net loss per share attributable to ordinary shareholders, basic and diluted	$ (1.69)	$ (2.89)	$ (10.22)	$ (4.48)
Weighted average number of ordinary shares outstanding, basic and diluted	34,864,911	9,180,759	22,559,389	8,872,768

Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	December 31,	
	2018	**2017**
Assets		
Current assets:		
Cash	$ 335,844	$ 89,856
Trade and other receivables	2,153	1,247
Prepaid expenses and other current assets	6,935	2,247
Research and development tax credit receivable	10,585	871
Total current assets	355,517	94,221
Non-current assets:		
Property and equipment, net	5,476	2,713
Restricted cash	3,837	—
Other long-term assets	1,212	360
Total non-current assets	10,525	3,073
Total assets	$ 366,042	$ 97,294
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 18,125	$ 3,891
Accrued expenses and other current liabilities	29,780	6,864
Total current liabilities	47,905	10,755
Other long-term liabilities	6,799	134
Total liabilities	54,704	10,889
Shareholders’ equity:	311,338	86,405
Total liabilities and shareholders’ equity	$ 366,042	$ 97,294

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