Orchard Therapeutics Reports Second Quarter 2019 Financial Results and Builds Momentum Ahead of Regulatory Filings

August 8, 2019

Metachromatic Leukodystrophy (MLD) MAA Submission and Initiation of ADA-SCID Rolling BLA Expected in the First Half of 2020; OTL-103 Recently Granted RMAT Designation for Wiskott-Aldrich Syndrome (WAS)

MLD and Mucopolysaccharidosis Type I (MPS-I) Abstracts Accepted for Oral Presentations at SSIEM 2019 Symposium

Ended the Second Quarter of 2019 with Approximately $423M in Total Cash and Investments; Recent Equity Financing of $130M Extends Cash Runway into the Second Half of 2021

Conference Call Scheduled for Today at 8:00 a.m. ET

BOSTON and LONDON, Aug. 08, 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 announced financial results and business highlights for the quarter ended June 30, 2019, as well as upcoming 2019 data presentations and milestones.

“In the first half of 2019, we made tremendous progress advancing our three lead programs toward regulatory filings, while pursuing a broader range of clinical opportunities for the hematopoietic stem cell-based gene therapy platform,” said Mark Rothera, president and chief executive officer of Orchard. “To deliver on our mission of bringing potentially curative therapies to patients around the world, we are expanding our global presence and preparing the ground for supply and access. If approved, we believe our therapies have the potential to provide a lifetime of benefit to patients in a single administration.”

Second Quarter and Recent Business Achievements

- Received regenerative medicine advanced therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) for OTL-103 for the treatment of Wiskott-Aldrich Syndrome (WAS). RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising advanced therapy products, including gene therapies. The OTL-103 program is continuing to enroll patients in a clinical trial using a cryopreserved formulation to support regulatory filings in the U.S. and Europe in 2021. Link to the full RMAT release here.
- Completed an underwritten public offering of 9,725,268 American Depositary Shares in June 2019 resulting in proceeds, net of underwriting discounts and commissions, of approximately $129.7 million. Link to the full release here.
- Signed a collaboration agreement with Fondazione Telethon and Ospedale San Raffaele for a new clinical-stage gene therapy program in mucopolysaccharidosis type I (MPS-I) showing encouraging preliminary data in an ongoing proof-of-concept trial. The trial is expected to enroll up to eight patients by the first half of 2020. Link to the full release here.
- Held a positive Marketing Authorization Application (MAA) pre-submission meeting with the European Medicines Agency (EMA) in early May 2019 for OTL-200 for metachromatic leukodystrophy (MLD). The company has brought forward the timeline for the planned submission of an MAA to the first half of 2020 and also expects to submit a Biologics License Application (BLA) in the U.S. approximately one year after the MAA submission.

Upcoming Data Presentations & Remaining 2019 Milestones

Neurometabolic Disorders

- Present data from the ongoing proof-of-concept trial of OTL-203 for MPS-I at the Society for the Study of Inborn Errors of Metabolism (SSIEM) 2019 Symposium on September 4, 2019 in Rotterdam, The Netherlands.
- Present an integrated data analysis of OTL-200 for MLD at the SSIEM 2019 Symposium on September 4, 2019 in Rotterdam, The Netherlands. The integrated analysis includes the 20 patient registrational data set in addition to data from expanded access patients.
- Report engraftment data in the first three patients from the ongoing clinical trial of OTL-200 for MLD using a cryopreserved

formulation.
- 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

- Report engraftment data in 10 patients from a clinical trial of OTL-101 for severe combined immune deficiency due to adenosine deaminase deficiency (ADA-SCID) using a cryopreserved formulation.
- Report three-year follow-up data in eight patients from a registrational trial of OTL-103 for WAS.
- Design and engage regulators on a planned registrational trial for OTL-102 for patients with X-linked chronic granulomatous disease (X-CGD).

Second Quarter 2019 Financial Results and Updated Cash Runway

Cash and investments as of June 30, 2019 were $423.4 million compared to $339.7 million as of December 31, 2018. The increase was primarily driven by proceeds from the company's public equity offering in June 2019, partially offset by the cash used to fund operations in the six months ended June 30, 2019.

Research and development expenses were $40.5 million for the three months ended June 30, 2019, compared to $151.0 million in the same period in 2018. The higher research and development expenses in 2018 resulted from a $133.6 million in-process research and development charge under the GSK agreement signed in April 2018. Excluding this one-time charge, research and development expenses would have increased $23.1 million for the three months ended June 30, 2019, compared to the same period in 2018. Research and development expenses for the three months ended June 30, 2019 include the upfront consideration for the license of the MPS-I clinical-stage program, as well as higher program and personnel-related costs from a larger portfolio compared to the corresponding period in 2018.

Selling, general and administrative expenses were $13.7 million for the three months ended June 30, 2019, compared to $7.4 million in the same period in 2018. The increase was primarily due to higher personnel costs to support public company operations as well as costs to prepare for the potential commercialization of the company's three late-stage development programs.

Net loss attributable to ordinary shareholders was $50.5 million for the three months ended June 30, 2019, compared to $156.2 million in the same period in 2018. The decrease as compared to the prior year was primarily due to the GSK agreement signed in April 2018. The company had 96.2 million ordinary shares outstanding as of June 30, 2019.

The company expects that its existing cash 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 the second quarter results and recent and upcoming business activities. To participate in the conference call, please dial 1-866-930-5155 (domestic) or 1-409-937-8974 (international) and refer to conference ID 9395864. 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 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 *ex vivo*, autologous, hematopoietic stem cell (HSC) based gene therapies includes Strimvelis®, a gammaretroviral vector-based gene therapy and 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 are all based on lentiviral vector-based gene modification of autologous HSCs and 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), transfusion-dependent beta-thalassemia (TDT) and mucopolysaccharidosis Type I (MPS-I), as well as an extensive preclinical pipeline. Strimvelis, as well as the programs in MLD, WAS and TDT were acquired by Orchard from GSK in April 2018 and originated from a pioneering collaboration between GSK and the San Raffaele Telethon Institute for Gene Therapy in 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 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," "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 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 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 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.

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.

Condensed Consolidated Statements of Operations
(In thousands, except share and per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Costs and operating expenses:				
Research and development	$ 40,478	$ 150,991	$ 57,971	$ 160,162
Selling, general and administrative	13,674	7,421	24,464	11,948
Total costs and operating expenses	54,152	158,412	82,435	172,110
Loss from operations	(54,152)	(158,412)	(82,435)	(172,110)
Other income (expense), net	2,850	2,097	987	401
Net loss before income tax	(51,302)	(156,315)	(81,448)	(171,709)
Income tax (expense) benefit	772	82	179	165
Net loss attributable to ordinary shareholders	(50,530)	(156,233)	(81,269)	(171,544)
Net loss per share attributable to ordinary shareholders, basic and diluted	$ (0.56)	$ (15.45)	$ (0.92)	$ (16.99)
Weighted average number of ordinary shares outstanding, basic and diluted	89,712,916	10,115,335	88,369,311	10,095,863

Condensed Consolidated Balance Sheets
(In thousands)
(unaudited)

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 132,783	$ 335,844
Marketable securities	286,748	—
Trade and other receivables	1,956	2,153
Prepaid expenses and other assets	5,143	6,935
Research and development tax credit receivable, current	10,594	10,585
Total current assets	437,224	355,517
Property and equipment, net	5,549	5,476
Research and development tax credit receivable	9,731	—
Restricted cash	3,843	3,837
Other long-term assets	1,770	1,212
Total assets	$ 458,117	$ 366,042
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 28,861	$ 18,125
Accrued expenses and other current liabilities	26,574	29,780
Total current liabilities	55,435	47,905

Long-term debt, net	24,501	—
Other long-term liabilities	7,024	6,799
Total liabilities	86,960	54,704
Total shareholders' equity	371,157	311,338
Total liabilities and shareholders' equity	$ 458,117	$ 366,042

Investors

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