Orchard Therapeutics Advances New Strategic Plan and Reports Second Quarter 2020 Financial Results

August 6, 2020

Regulatory and Clinical Progress in MLD, MPS-I and MPS-IIIA Illustrate Execution in Neurometabolic Disorders; EU Regulatory Decision for Libmeldy ™Expected in 2H 2020

MolMed and GSK Agreements Provide Near-term Manufacturing Capacity and Highlight Future Innovations

Near-term Financial Position Secure with Cash and Investments of ~$230M and Runway into 2022

BOSTON and LONDON, Aug. 06, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today reviewed business updates and upcoming milestones supporting its new strategic plan and reported financial results for the quarter ended June 30, 2020.

“Implementing Orchard’s new strategic plan yielded strong results in the second quarter as we marked important regulatory and clinical progress in our neurometabolic programs and strengthened our current and future manufacturing position,” said Bobby Gaspar, M.D., Ph.D., chief executive officer of Orchard. “Our commercial infrastructure and launch plans continue to develop with a focus on site qualification, diagnostic initiatives, pricing and reimbursement as we prepare for the potential approval of Libmeldy (formerly OTL-200) in the EU. Towards the end of the year, we plan to share more detail from our new research programs in FTD and Crohn’s disease, which we believe highlight the potential for HSC gene therapy beyond ultra-rare diseases.”

Recent Corporate Achievements Supporting New Strategic Plan

Prioritize portfolio investments to realize opportunities for high need, high value indications, with an emphasis on neurometabolic conditions

- **Libmeldy (OTL-200) for MLD:**
  - Submitted responses to the Day 120 questions from the European Medicines Agency (EMA) for the marketing authorization application (MAA) for Libmeldy (OTL-200) for the treatment of metachromatic leukodystrophy (MLD).
- **OTL-203 for MPS-I:**
  - Received orphan drug and rare pediatric disease designations for the treatment of mucopolysaccharidosis type I (MPS-I) from the U.S. Food and Drug Administration (FDA).
  - Presented interim data from the ongoing proof-of-concept trial of OTL-203 at the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting. The first primary outcome measure of the study was met with all eight patients achieving hematologic engraftment within 45 days of treatment. In addition, clinical data from the first two patients treated have shown improved motor function, stable cognitive scores and continued normal growth after at least one year of follow-up. Link to full release here.
- **OTL-201 for MPS-IIIA:**
  - Enrolled three patients in the proof-of-concept study of OTL-201 for the treatment of mucopolysaccharidosis type IIIA (MPS-IIIA).

Establish focused commercial model for diagnosis and treatment of patients globally

- Extended the gene therapy manufacturing relationship with MolMed S.p.A (an AGC Inc. company) for a period of five years through June 2025. MolMed will continue to support activities related to the development and manufacturing of vectors and drug product for multiple Orchard investigational programs, including for the potential upcoming commercial launch of Libmeldy (OTL-200) for MLD. Link to full release here.

Invest in next-generation manufacturing technology and process innovations

- Entered into worldwide royalty-bearing license agreements with GlaxoSmithKline plc (GSK) for use of their proprietary lentiviral stable cell line technology in gene therapy products for the treatment of Wiskott Aldrich syndrome (WAS) and transfusion-dependent beta thalassemia (TDT). Link to full release here.

Planned Corporate Milestones

A Media Snippet accompanying this announcement is available by clicking on the image or link below:

Orchard Therapeutics: Media Snippet

Second Quarter 2020 Financial Results

Frank Thomas, president and chief operating officer added, "We have successfully implemented many of the operational changes under our new strategic plan on schedule, including the decommissioning of the California site. We are seeing the financial benefits of a streamlined footprint and organization heading into the second half of 2020 with a disciplined approach to investing in the high value programs we prioritized under the new plan."

Research and development expenses were $31.6 million for the second quarter of 2020 compared to $40.5 million in the same period in 2019. Research and development 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. R&D expenses in 2019 included a one-time $17.2 million in-process research and development charge associated with the in-licensing of our OTL-203 program for the treatment of MPS-I. In 2020, R&D expenses included a non-cash restructuring charge of $5.6 million related to the consolidation and closure of our R&D and manufacturing facilities in California. Excluding these charges, R&D expenses increased by $2.7 million from $23.3 million in the second quarter of 2019 to $26.0 million in the second quarter of 2020.

Selling, general and administrative expenses were $15.7 million for the second quarter of 2020 compared to $13.7 million in the same period in 2019. The increase was primarily due to increased launch readiness activities, personnel-related costs and share-based compensation expense.

Net loss was $47.5 million for the second quarter of 2020 compared to $50.5 million in the same period in 2019. The decrease was primarily due to the in-process research and development expense for the MPS-I license deal signed in May 2019, offset by increases in R&D and SG&A expenses. The company had 97.5 million ordinary shares outstanding as of June 30, 2020.

Cash, cash equivalents and investments as of June 30, 2020, were $228.7 million compared to $325.0 million as of December 31, 2019. The decrease was primarily driven by cash used to fund operations in the first half of 2020. 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.

Conference Call & Webcast Information

Orchard will host a conference call and live webcast today at 8:00 a.m. ET to discuss its second quarter business highlights and financial results. The conference call will be broadcast live in listen-only mode 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 presentation. To ask a question, please dial (866) 987-6504 (U.S. domestic) or +1 (602) 563-8620 (international) and refer to conference ID 7392139. Please dial in at least 15 minutes in advance to ensure a timely connection to the call.

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, the therapeutic potential of Orchard's product candidates, including the 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, Orchard's expectations concerning its partnership with MolMed, Orchard's expectations regarding its expected use of stable cell line technology and the potential benefits of its agreements with GSK, 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 severity of the impact of the COVID-19 pandemic on Orchard's business, including on clinical development, its supply chain and commercial programs; the risk that Orchard will not realize the anticipated benefits of its new strategic plan or the expected cash savings associated

with such plan; 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 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 or the receipt of restricted marketing approvals; the risk of delays in Orchard's ability to commercialize its product candidates, if approved; and the risk that the market opportunity for its product candidates may be lower than estimated. 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 June 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Product sales, net	$ 597	$ —	$ 597	$ —
Costs and operating expenses:				
Cost of product sales	191	—	191	—
Research and development	31,568	40,478	$ 56,404	57,971
Selling, general and administrative	15,659	13,674	35,804	24,464
Total costs and operating expenses	47,418	54,152	92,399	82,435
Loss from operations	(46,821)	(54,152)	(91,802)	(82,435)
Other income (expense):				
Interest income	892	1,727	2,372	3,350
Interest expense	(568)	(245)	(1,181)	(245)
Other income (expense), net	(943)	1,368	(7,733)	(2,118)
Total other income (expense), net	(619)	2,850	(6,542)	987
Net loss before income tax	(47,440)	(51,302)	(98,344)	(81,448)
Income tax (expense) benefit	(60)	772	275	179
Net loss attributable to ordinary shareholders	(47,500)	(50,530)	(98,069)	(81,269)
Net loss per share attributable to ordinary shareholders, basic and diluted	$ (0.48)	$ (0.56)	$ (0.99)	$ (0.92)
Weighted average number of ordinary shares outstanding, basic and diluted	99,251,314	89,712,916	99,048,498	88,369,311

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

	June 30, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 228,654	$ 324,990
Trade receivables	723	1,442
Research and development tax credit receivable	32,729	28,644
Prepaid expenses and other current assets	12,760	8,530
Operating lease right-of-use assets	18,642	19,415
Property and equipment, net	5,460	7,596
Other assets	15,608	8,664

Total assets	$ 314,576	$ 399,281
Liabilities and shareholders' equity		
Accounts payable	$ 15,590	$ 11,984
Accrued expenses and other current liabilities	19,811	37,980
Operating lease liabilities	23,095	21,212
Long-term debt, net	24,886	24,699
Other long-term liabilities	4,173	4,213
Total liabilities	87,555	100,088
Shareholders’ equity	227,021	299,193
Total liabilities and shareholders’ equity	$ 314,576	$ 399,281

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