



Orchard Therapeutics Unveils New Strategic Plan and Reports First Quarter 2020 Financial Results

May 7, 2020

Portfolio Investments More Tightly Prioritized Around High Need, High Value Indications

First Disease Targets Extending Platform into Less Rare Indications Disclosed

Updated Timelines Provided for MLD and MPS-I Programs

California Facility Investment Terminated with Near-term Manufacturing Capacity Secured

Expected Savings of Approximately \$125M, Extending Cash Runway into 2022

BOSTON and LONDON, May 07, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today announced a new strategic plan to realize the potential of the hematopoietic stem cell (HSC) gene therapy approach and reported its financial results for the quarter ended March 31, 2020. The plan outlined by the new leadership team is built around strategic priorities designed to achieve sustainable value creation.

"I feel privileged to lead Orchard as we embark on this new chapter, which is rooted in fulfilling the powerful possibilities for HSC gene therapies beyond ultra-rare diseases," said Bobby Gaspar, M.D., Ph.D., chief executive officer. "Moving forward, we are focusing on advancing therapies for high need and high value diseases, and our work in neurometabolic disorders is a clear example of this. We're also excited to announce new research programs which we believe will demonstrate the breadth of the HSC platform approach."

Core Elements of the New Strategic Plan

Realizing the potential of the HSC gene therapy approach

- 1. Prioritize portfolio investments to realize opportunities for high need, high value indications**
- 2. Establish focused commercial model for diagnosis and treatment of patients globally**
- 3. Invest in next-generation manufacturing technology and process innovations**
- 4. Create operational efficiencies to maintain financial strength and flexibility**

The company is announcing several key changes to its operations intended to support the new strategic plan. These include plans to:

- Establish MLD (OTL-200), WAS (OTL-103), MPS-I (OTL-203) and MPS-IIIA (OTL-201) programs as top near-term priorities and reduce investment in ADA-SCID (OTL-101) and TDT (OTL-300);
- Accelerate research in less rare indications, including two new programs in genetic subsets of frontotemporal dementia (FTD) and Crohn's disease, announced today;
- Phase the commercial build to align with expected launch trajectories for OTL-200, which focuses primarily on the incidence-based opportunity, and OTL-103, which provides a significantly prevalence-based opportunity; and
- Focus manufacturing strategy by prioritizing investments in technology and process innovations, closing the company's California site, including the termination of the Fremont project and associated capital, and phasing investment in future manufacturing capacity.

As a result of these decisions, the company has reduced its current headcount by approximately 25%. Collectively, all of the actions announced today are expected to achieve cash savings of approximately \$125 million through the end of 2021, extending the company's existing cash runway into 2022.

The company also provided an update on the progress of its U.S. biologics license application (BLA) submission of OTL-200 for the treatment of MLD. The U.S. Food and Drug Administration (FDA) recently provided written feedback on the sufficiency of the company's data package, including the clinical endpoints, natural history analysis and chemistry, and manufacturing and controls (CMC) data. Orchard intends to use FDA's guidance to further analyze the latest available CMC and clinical data, file an investigational new drug (IND) application and seek Regenerative Medicine Advanced Therapy (RMAT) designation in 2020. The company believes this will facilitate a more comprehensive dialogue with the FDA to resolve open matters before the intended BLA submission.

"Our new strategic plan positions Orchard to execute its mission and objectives at the highest level by matching our attention and resources to a set of

core imperatives for the business,” said Frank Thomas, president and chief operating officer. “I believe that these are necessary steps, especially in light of the current environment in which we are operating, with focused investments in areas such as commercial and manufacturing operations supporting the needs we have now without a near-term dependence on the capital markets.”

Upcoming Expected Corporate Milestones

Metachromatic Leukodystrophy (MLD)

- Obtain approval in the EU for OTL-200 for the treatment of MLD in the second half of 2020 and launch in the first half of 2021
- File an IND and seek RMAT designation in the U.S. for OTL-200 for the treatment of MLD in 2020, with the intention of submitting a BLA pending the resolution of FDA's feedback

Wiskott-Aldrich syndrome (WAS)

- Prepare for BLA and marketing authorization application (MAA) regulatory filings in the U.S. and EU, respectively, for OTL-103 for the treatment of WAS in 2021

Mucopolysaccharidosis Type I (MPS-I)

- Report interim data from the proof-of-concept study of OTL-203 for the treatment of MPS-I in the second half of 2020
- Report one-year follow-up results for OTL-203 for the treatment of MPS-I, including the primary endpoints, in the first half of 2021
- Initiate a registrational study for OTL-203 for the treatment of MPS-I in 2021

Mucopolysaccharidosis Type IIIA (MPS-IIIA)

- Enroll five patients and report interim data from the proof-of-concept study of OTL-201 for the treatment of MPS-IIIA in 2021

First Quarter 2020 Financial Results

Research and development expenses were \$24.8 million for the first quarter of 2020 compared to \$17.5 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.

Selling, general and administrative expenses were \$20.1 million for the first quarter of 2020 compared to \$10.8 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, including a one-time non-cash charge associated with the departure of the company's former chief executive officer.

Net loss was \$50.6 million for the first quarter of 2020 compared to \$30.7 million in the same period in 2019. The increase was primarily due to higher operating costs to support later phases of development across the gene therapy portfolio and activities to prepare for commercialization of the company's most advanced programs. The company had 97.2 million ordinary shares outstanding as of March 31, 2020.

Cash, cash equivalents and investments as of March 31, 2020, were \$263.9 million compared to \$325.0 million as of December 31, 2019. The decrease was primarily driven by cash used to fund operations of \$49.9 million in the first quarter of 2020. Following the actions announced today, the company now 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 with slides today at 8:00 a.m. ET to discuss its new corporate strategy. 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 8348144. 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.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, 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, the size of the potential markets for Orchard's product candidates, the adequacy of the company's manufacturing capacity and plans for future investment, and the company'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 and commercial programs; the risk that Orchard will not realize the anticipated benefits of its new strategic plan or the expected cash savings of approximately \$125 million through the end of 2021; 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; 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 annual report on Form 10-K for the year ended December 31, 2019, as filed with the U.S. Securities and Exchange Commission (SEC) on February 27, 2020, 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 Data (in thousands, except share and per share data) (Unaudited)

	Three Months Ended March 31,	
	2020	2019
Costs and operating expenses:		
Research and development	\$ 24,836	\$ 17,493
Selling, general and administrative	20,145	10,790
Total costs and operating expenses	44,981	28,283
Loss from operations	(44,981)	(28,283)
Other income (expense):		
Interest income	1,480	1,623
Interest expense	(613)	—
Other income (expense), net	(6,790)	(3,486)
Total other income (expense), net	(5,923)	(1,863)
Net loss before income tax	(50,904)	(30,146)
Income tax (expense) benefit	335	(593)
Net loss attributable to ordinary shareholders	(50,569)	(30,739)
Net loss per share attributable to ordinary shareholders, basic and diluted	\$ (0.51)	\$ (0.35)
Weighted average number of ordinary shares outstanding, basic and diluted	98,713,126	87,010,596

Condensed Consolidated Balance Sheet Data
(in thousands)
(Unaudited)

	March 31	December 31,
	2020	2019
Assets		
Cash, cash equivalents and marketable securities	\$ 263,865	\$ 324,990
Trade receivables	709	1,442
Research and development tax credit receivable	30,211	28,644
Prepaid expenses and other current assets	19,547	8,530
Operating lease right-of-use assets	21,932	19,415
Property and equipment, net	8,679	7,596
Other assets	9,904	8,664
Total assets	\$ 354,847	\$ 399,281
Liabilities and shareholders' equity		
Accounts payable	\$ 13,479	\$ 11,984
Accrued expenses and other current liabilities	25,122	37,980
Operating lease liabilities	23,694	21,212
Long-term debt, net	24,794	24,699
Other long-term liabilities	3,204	4,213
Total liabilities	90,293	100,088
Shareholders' equity:	264,554	299,193
Total liabilities and shareholders' equity	\$ 354,847	\$ 399,281

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Source: Orchard Therapeutics (Europe) Limited