Orchard Therapeutics Outlines Business Impact of COVID-19

March 31, 2020

BOSTON and LONDON, March 31, 2020 (GLOBE NEWSWIRE) -- Orchard Therapeutics (Nasdaq: ORTX), a global leader in gene therapy, today provided an update on its business and operations, outlining how the COVID-19 pandemic has and may continue to impact the company's development programs and timelines, product supply, commercial operations and financial position. Orchard has taken important steps to help ensure the safety of employees and their families and reduce spread of the virus in the communities where the company operates.

"We are operating in an unprecedented time for our business and the industry as a whole, given the rapid and global spread of COVID-19," said Bobby Gaspar, chief executive officer of Orchard. "During this challenging period, I have seen our employees develop creative solutions to issues posed by the current situation. We are in a strong financial position with a geographically dispersed workforce, and I am confident in our ability to swiftly and nimbly adapt and be proactive with the goal of minimizing further disruptions to our business during this time."

While the company continues to progress its development, regulatory and commercialization plans, it also acknowledges the following impacts of COVID-19 on clinical activities, regulatory timelines and commercial readiness efforts that are underway.

Regulatory and commercialization timelines of most advanced programs

- OTL-200 for metachromatic leukodystrophy (MLD)
  • Orchard continues to engage with the European Medicines Agency (EMA) on the company's marketing authorization application (MAA) for OTL-200 for the treatment of MLD under an accelerated assessment. While the company is still preparing for a potential approval in 2020, the timeline could be extended as a result of the coordination needed among EMA, the company, its clinical site, its manufacturing partners and other key stakeholders involved in the review process during this time.
  • Throughout the remainder of 2020, Orchard plans to continue its commercial preparation efforts in Europe, which include a focus on patient identification, disease awareness, site qualification and market access activities. If approved, the commercial launch of OTL-200 in Europe is likely to occur in the first half of 2021 based on expected regulatory timelines.
  • In addition, the company now believes it is likely to submit the biologics license application (BLA) for MLD with the U.S. Food and Drug Administration (FDA) in the first half of 2021 due to COVID-19 related impacts.

- OTL-101 for adenosine deaminase severe combined immunodeficiency (ADA-SCID)
  • Based on COVID-19-related impacts at Orchard's clinical and manufacturing sites, the company does not expect to complete the necessary activities to enable initiation of its rolling BLA in the U.S. in the first half of 2020.

- OTL-103 for Wiskott-Aldrich syndrome (WAS)
  • The U.S. and EU regulatory filings for the OTL-103 program in WAS remain on track for 2021. The company will continue to monitor the program's progression of clinical and regulatory activities in light of COVID-19, including any impact on filing timelines.

Clinical trial enrollment and follow-up

- Orchard's OTL-203 clinical program for the treatment of mucopolysaccharidosis type I (MPS-I) has completed enrollment in a proof-of-concept study, and interim data is still expected to be released in the second half of 2020.
- Additionally, while the company's clinical sites are still treating patients in studies for OTL-201, OTL-103 and OTL-200 (for the treatment of mucopolysaccharidosis type IIIA (MPS-IIIA), WAS and MLD, respectively), these centers are devoting significant resources to patients with COVID-19, which could limit their ability to enroll additional patients in ongoing clinical studies. While the company believes it has enrolled and treated enough patients to support regulatory filings for OTL-200 in the U.S. and Europe, COVID-19-related impacts are likely to shift the enrollment timelines of the OTL-201 and OTL-103 trials by at least three months.
- Follow-up visits associated with all active Orchard clinical trials are likely to be conducted using alternative data collection

approaches due to COVID-19 travel and other trial site limitations. Orchard is closely evaluating recently issued regulatory guidance and attempting to capture the necessary patient data to support future regulatory filings.

Access to Strimvelis®

- Ospedale San Raffaele, Milan, Italy, the treatment site for Strimvelis, the first autologous *ex vivo* gene therapy approved by EMA for ADA-SCID, has postponed scheduling and treating non-urgent patients with the therapy. Individuals who have been identified as qualifying patients will continue to receive enzyme replacement therapy until treatment with Strimvelis can occur.

Supply chain and infrastructure

- Orchard is working closely with its contract manufacturers and third-party logistics providers to ensure patient supply. The company's key suppliers have remained operational for certain critical activities despite the COVID-19 situation. The company continues to monitor ongoing events and prioritize activities to mitigate potential interruptions.
- Construction activities on the company's planned manufacturing facility in Fremont, California, have been temporarily suspended as a result of the pandemic and the related "shelter-in-place" order affecting that region.

"We want to thank the many healthcare professionals around the world who are working tirelessly to fight the pandemic. At Orchard, our mission remains unchanged, and we will continue efforts on our path to deliver therapies to the patients we serve, while also doing our part to support the priorities of the global healthcare system during this period," continued Gaspar. "The impact of COVID-19 on our business has heightened our vigilance to review and refine our business plans and priorities, an assessment which was already underway as a result of the company's recent leadership transition."

The company plans to provide further updates on any incremental COVID-19-related impacts to its business operations, development programs and commercialization timelines when appropriate. Management intends to further communicate any changes to the operating plan on the company's first quarter conference call, which will include the results of a portfolio review and the status of Orchard's commercial readiness activities for OTL-200.

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. Forward-looking statements include express or implied statements relating to, among other things, Orchard's business strategy, goals and outlook, as well as Orchard's plans and expectations in light of and in response to the COVID-19 global pandemic, including its impacts on trial enrollment for and timing of Orchard's ongoing clinical trials, expectations relating to regulatory submissions for Orchard's product candidates, and expectations regarding potential regulatory approvals. 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 timing of clinical trials and regulatory applications and submissions for Orchard's product candidates, including those candidates described in this press release, and the treatment of patients with Orchard's commercial product, Strimvelis®; the risk that any one or more of Orchard's product candidates 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 failure to obtain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates or 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 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.

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