Orchard Therapeutics Announces the Filing and Validation of Marketing Authorization Application by European Medicines Agency for OTL-200 for the Treatment of Metachromatic Leukodystrophy

December 2, 2019

Application Being Evaluated Under Accelerated Assessment

BOSTON and LONDON, Dec. 02, 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 that the European Medicines Agency (EMA) has validated the company's Marketing Authorization Application (MAA) for OTL-200, an *ex vivo,* autologous, hematopoietic stem cell-based gene therapy that has been developed in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy, for the treatment of metachromatic leukodystrophy (MLD). Validation of the MAA confirms that the submission is sufficiently complete to begin the formal review process.

"We are pleased that OTL-200 for the treatment of MLD is now under review with the EMA, bringing us another step closer to potentially making an approved gene therapy treatment a reality for children and families affected by this devastating and rapidly progressing disease," said Mark Rothera, president and chief executive officer of Orchard Therapeutics. "We are committed to working with the EMA as they evaluate our application. Due to the nature of the disease and the urgency to treat those affected by MLD, we have also been working diligently in parallel to make OTL-200, if approved, available to patients in the EU as quickly as possible, while continuing our efforts to expand patient access outside the EU."

Orchard previously announced in November 2019 that the EMA had granted accelerated assessment for OTL-200. Accelerated assessment potentially provides a reduced review timeline from 210 to 150 days once the MAA is filed and validated, not counting clock stops when applicants are requested to provide additional information.

About MLD and OTL-200
Metachromatic leukodystrophy (MLD) is a rare and life-threatening inherited disease of the body's metabolic system occurring in approximately one in every 100,000 live births. MLD is caused by a mutation in the *arylsulfatase-A* (*ARSA*) gene that results in the accumulation of sulfatides in the brain and other areas of the body, including the liver, the gallbladder, kidneys, and/or spleen. Over time, the nervous system is damaged and patients with MLD will experience neurological problems such as motor, behavioral and cognitive regression, severe spasticity and seizures, finding it more and more difficult to move, talk, swallow, eat and see. Currently, there are no effective treatments for MLD. In its late infantile form, mortality at 5 years from onset is estimated at 50% and 44% at 10 years for juvenile patients.[1] OTL-200 is an *ex vivo*, autologous, hematopoietic stem cell-based gene therapy being studied for the treatment of MLD. OTL-200 was acquired from GSK in April 2018 and originated from a pioneering collaboration between GSK and the Hospital San Raffaele and Fondazione Telethon, acting through their joint San Raffaele-Telethon Institute for Gene Therapy in Milan, initiated in 2010.

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, including the product candidate or 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, including the product candidate or candidates referred to in this release, the timing of announcement of clinical data for its product candidates, including the product candidate or candidates referred to in this release, and the likelihood that such data will be positive and support further clinical development and regulatory approval of its product candidates, and the likelihood of approval of such product candidates by the applicable regulatory authorities. 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: 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, including the product candidate or candidates referred to in this release, 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.

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[1] Mahmood et al. Metachromatic Leukodystrophy: A Case of Triplets with the Late Infantile Variant and a Systematic Review of the Literature. Journal of Child Neurology 2010, DOI: http://doi.org/10.1177/0883073809341669

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