



European Medicines Agency Grants Orchard Therapeutics Accelerated Assessment of OTL-200 for Patients with Metachromatic Leukodystrophy

November 18, 2019

BOSTON and LONDON, Nov. 18, 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 Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has granted an accelerated assessment for OTL-200, a gene therapy in development for the treatment of metachromatic leukodystrophy (MLD) in partnership with the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) in Milan, Italy.

"We are pleased that OTL-200 has been granted accelerated assessment and believe this underscores the quality of our clinical data and the urgent need to bring this novel, potentially curative treatment option to patients living with MLD," said Anne Dupraz-Poiseau, Ph.D., chief regulatory officer of Orchard Therapeutics. "We look forward to working with the EMA to ensure this potentially transformative new treatment, if approved, reaches patients in the EU as quickly as possible, and continuing our efforts to expand patient access outside the EU."

The EMA awards an accelerated assessment to medicines that are expected to be of major public health interest, particularly in the area of therapeutic innovation. Accelerated assessment potentially provides a reduced review timeline from 210 to 150 days once the Marketing Authorization Application (MAA) is filed and validated, not counting clock stops when applicants are requested to provide additional information. The decision to grant accelerated assessment has no impact on the eventual CHMP and Committee for Advanced Therapies (CAT) opinion on whether a marketing authorization should be granted.

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, 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.

¹Mahmood 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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Source: Orchard Therapeutics (Europe) Limited