Orchard Therapeutics' OTL-200 Receives Rare Pediatric Disease Designation from FDA for Treatment of Metachromatic Leukodystrophy

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Orchard's fourth Rare Pediatric Disease Designation for autologous *ex vivo* gene therapy

BOSTON and LONDON, May 3, 2018 – Orchard Therapeutics, a leading commercial stage company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted a Rare Pediatric Disease Designation to the company's gene therapy candidate OTL-200, for the treatment of patients with metachromatic leukodystrophy (MLD).

MLD is a rare, fatal, neurodegenerative, inherited metabolic disease caused by mutations in the ARSA gene. In its late infantile and juvenile forms, which represents the majority of MLD patients, mortality at 5 years is estimated at 75% and 30%, respectively[1].

The FDA grants Rare Pediatric Disease Designations for serious or life-threatening diseases with manifestations in individuals aged from birth to 18 years, including access to the FDA's expedited review and approval process. The Rare Pediatric Disease Designation makes the program eligible for a Rare Pediatric Disease Priority Review Voucher upon approval of OTL-200 by the FDA. OTL-200 was acquired by Orchard from GSK in April 2018 and originated from a pioneering collaboration between GSK and the Hospital San Raffaele and the Telethon Foundation, acting through their joint Telethon Institute for Gene Therapy, in Milan, initiated in 2010. This collaboration led to the development of Strimvelis, the world's first approved autologous *ex vivo* gene therapy product. Orchard anticipates filing OTL-200 for market authorization with regulatory authorities from 2019.

"This is the fourth Rare Pediatric Disease Designation awarded by the FDA to one of Orchard's autologous *ex vivo* gene therapy candidates, providing significant incentives to continue expanding our pipeline" commented Mark Rothera, president and CEO of Orchard "MLD is a devastating disease in which most patients do not survive the first decade of life. In clinical trials, early treatment with OTL-200 has demonstrated preservation of cognitive and motor development to levels comparable with healthy individuals[2]. We look forward to working with Telethon / Ospedale San Raffaele to bring this potentially life-changing treatment to patients as rapidly as possible."

About Orchard

Orchard Therapeutics is a leading global fully integrated commercial stage company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies.

Orchard's portfolio of autologous *ex vivo* gene therapy programs has demonstrated sustained clinical benefit in over 120 patients across five disease areas. These programs include Strimvelis, the first autologous *ex vivo* gene therapy approved by the EMA in 2016, 3 programs in advanced registrational studies in MLD (metachromatic leukodystrophy), WAS (Wiskott Aldrich syndrome) and ADA-SCID (adenosine deaminase severe combined immunodeficiency), other clinical programs in X-CGD (X-linked chronic granulomatous disease) and beta-thalassemia, as well as an extensive preclinical pipeline.

The company is partnered with world-leading institutions in gene therapy, including University College London, Great Ormond Street Hospital, the University of Manchester and Central Manchester University Hospitals, the University of California Los Angeles, Boston Children's Hospital, and Telethon Institute of Gene Therapy/Ospedale San Raffaele.

Orchard is privately held with offices in the UK and the US, including London, San Francisco and Boston. The company raised $110 million in a Series B in December 2017, was named a Fierce 15 Company by FierceBiotech in 2016 and was awarded a $19 million grant from the California Institute of Regenerative Medicine (CIRM).

For further information please visit www.orchard-tx.com

[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

[2]Sessa *et al*. Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. *The Lancet* 2016, DOI: http://dx.doi.org/ 10.1016/S0140-6736(16)30374-9

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