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Orchard Therapeutics' OTL-200 receives RPD Designation from FDA

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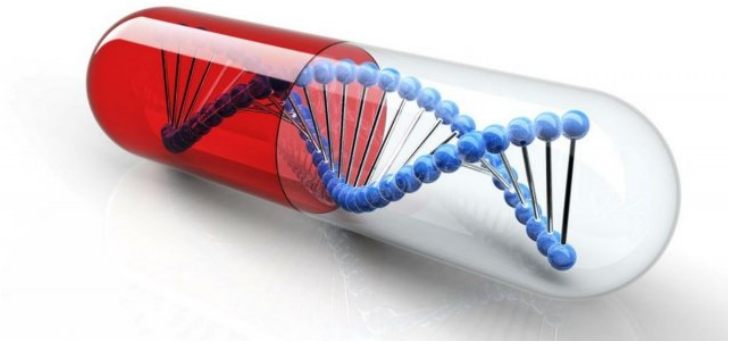
Orchard's fourth Rare Pediatric Disease Designation for autologous ex vivo gene therapy



Orchard Therapeutics, a leading commercial stage company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies, 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 represent the majority of MLD patients, mortality at 5 years is estimated at 75% and 30%, respectively.

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.