

## **OTL-200**

OTL 200 (formerly known as GSK 2696274), an ex vivo stem cell gene therapy is being developed by Orchard Therapeutics for the treatment of metachromatic leukodystrophy (An autosomal recessive inherited disorder characterized by abnormalities in the development of the myelin sheaths. It is caused by a deficiency of the enzyme arylsulfatase A. There are three forms of this disease: late infantile, juvenile, and adult. In the late infantile form symptoms include muscle weakness and rigidity, gait disturbances, developmental delays, and seizures. In the juvenile form symptoms include gait disturbances, mental deterioration and seizures. The adult form is characterized by psychotic symptoms and dementia.) . Orchard company is developing OTL 200 under a license from GlaxoSmithKline, Fondazione Telethon and Ospedale San Raffaele - Telethon Institute for Gene Therapy (OSR-TIGET).

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. Top of Form