First gene therapy for children with metachromatic leukodystrophy approved by FDA

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The U.S. Food and Drug Administration has approved Orchard Therapeutics' Lenmeldy (atidarsagene autotemcel) as the first gene therapy for the treatment of children with metachromatic leukodystrophy (MLD).

Lenmeldy is a one-time, individualized single-dose infusion made from a patient's own hematopoietic stem cells, which have been genetically modified to include functional copies of the ARSA gene that codes for the arylsulfatase A enzyme. It is indicated for presymptomatic late infantile, presymptomatic early juvenile, or early symptomatic early juvenile MLD.

The priority-review orphan drug approval was based on data from 37 children who received Lenmeldy. Compared with untreated children, Lenmeldy significantly reduced the risk for severe motor impairment or death.

All children with presymptomatic late infantile MLD treated with Lenmeldy were alive at 6 years of age versus only 58 percent of untreated children. Seven in 10 treated children (71 percent) were able to walk without assistance at 5 years of age, and 85 percent had normal language and performance IQ scores.

Children with presymptomatic early juvenile and early symptomatic early juvenile MLD showed slowing of motor and/or cognitive disease with Lenmeldy.

"MLD is a devastating disease that profoundly affects the quality of life of patients and their families. Advancements in treatment options offer hope for improved outcomes and the potential to positively influence the trajectory of disease progression," Nicole Verdun, M.D., director of the Office of Therapeutic Products at the FDA, said in a statement.

"This approval represents important progress in the advancement and availability of effective treatments, including gene therapies, for rare diseases."

More information: More Information