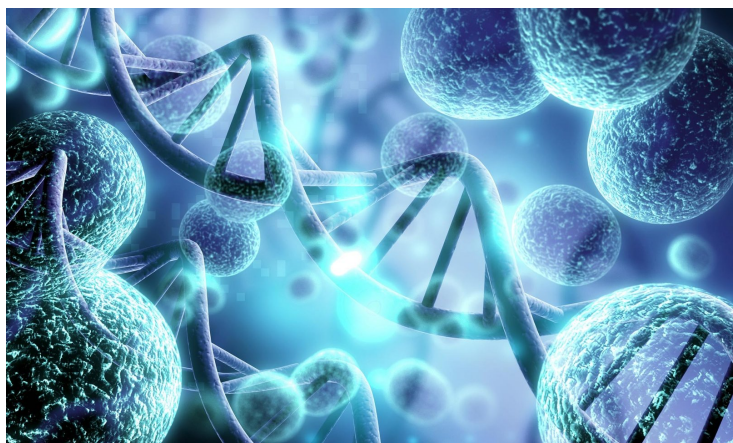


US FDA expands approval of gene therapy for patients with Duchenne Muscular Dystrophy

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Granted the approval and accelerated approval for Elevidys to Sarepta Therapeutics Inc.



The US Food and Drug Administration (FDA) has expanded the approval of Elevidys (delandistrogene moxeparvovec-rokl), a gene therapy for the treatment of Duchenne muscular dystrophy (DMD) for ambulatory and non-ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene.

Elevidys was previously approved under accelerated approval for ambulatory individuals 4 through 5 years of age with DMD with a confirmed mutation in the *DMD* gene.

With this new action, Elevidys received traditional approval in ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene, and accelerated approval in non-ambulatory individuals 4 years of age and older with DMD with a confirmed mutation in the *DMD* gene.

Duchenne muscular dystrophy is a rare and serious genetic condition which worsens over time, leading to weakness and wasting away of the body's muscles. The disease occurs due to a defective gene that results in abnormalities in, or absence of, dystrophin, a protein that helps keep the body's muscle cells intact.

Elevidys is a recombinant gene therapy designed to deliver into the body a gene that leads to production of Elevidys micro-dystrophin, a shortened protein (138 kDa, compared to the 427 kDa dystrophin protein of normal muscle cells) that contains selected domains of the dystrophin protein present in normal muscle cells. The product is administered as a single intravenous dose.