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Novartis gets FDA approval for gene therapy labeled at $2M

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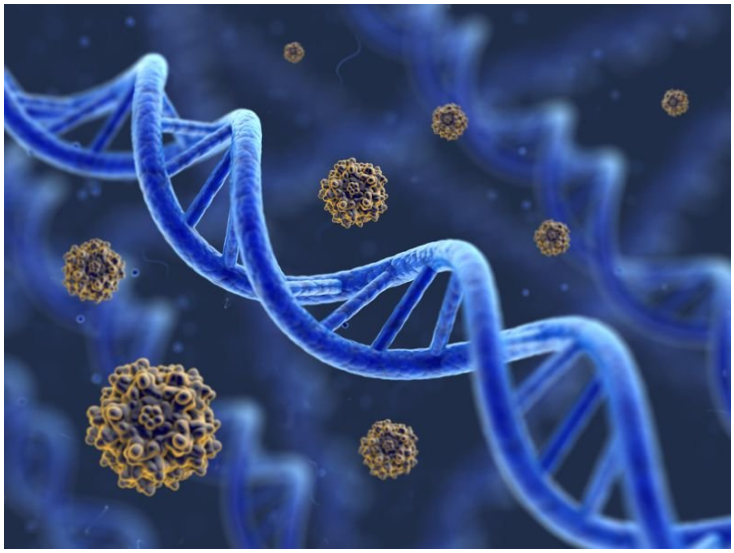
Zolgensma (onasemnogene abeparvovec-xioi) is approved for the treatment of pediatric patients less than 2 yearsof age with spinal muscular atrophy (SMA)



AveXis, a Novartis company, has announced the US Food and Drug Administration (FDA) has approved Zolgensma® (onasemnogene abeparvovec-xioi) for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (*SMN1*) gene.

Zolgensma is designed to address the genetic root cause of SMA by providing a functional copy of the human SMN gene to halt disease progression through sustained SMN protein expression with a single, one-time intravenous (IV) infusion.

Zolgensma is the first and only gene therapy approved by the FDA for the treatment of SMA, including those who are pre-symptomatic at diagnosis. This therapy comes with a price tag of $2.1 million, making it the most expensive drug.

SMA is a rare, genetic neuromuscular disease caused by a defective or missing *SMN1* gene. Without a functional *SMN1* gene, infants with SMA lose the motor neurons responsible for muscle functions such as breathing, swallowing, speaking and walking.

Zolgensma will be made available in the US and will be marketed by AveXis, a Novartis company. Outside of the US, Zolgensma has PRIME (PRIority MEdicines) designation in Europe and is being reviewed under Accelerated Assessment Procedure, and also has accelerated Sakigake designation in Japan.