US FDA approves Novartis' gene therapy for rare muscle disorder

The therapy, branded as Itvisma, was approved for the treatment of spinal muscular atrophy patients of age two years and older who have a confirmed mutation in the survival motor neuron 1 gene.

London: The U.S. Food and Drug Administration has approved Novartis' gene therapy for patients with a rare muscle disorder, the drugmaker said on Monday.

The therapy, branded as Itvisma, was approved for the treatment of spinal muscular atrophy patients of age two years and older who have a confirmed mutation in the survival motor neuron 1 gene.

Itvisma contains the same active ingredient as the Swiss drugmaker's older therapy, Zolgensma, which is approved in the U.S. to treat SMA patients less than 2 years of age.

The new treatment has a wholesale acquisition cost of $2.59 million, compared with $2.1 million for Zolgensma.

Itvisma is the first and only gene replacement therapy available for the broad population, Novartis said.

"(This) gives patients even more choice, which for any patient is a good thing," Tracey Dawson, U.S. Therapeutic Area Head of Neuroscience at Novartis, told Reuters ahead of the approval.

In a late-stage trial, treatment with Itvisma led to a statistically significant 2.39-point improvement on a scale that assesses motor ability and disease progression.

Spinal muscular atrophy is a rare, genetic neuromuscular disease caused by a mutated or missing SMN1 gene, which is responsible for the production of a protein needed for muscle function, including breathing, swallowing and basic movement.

It is the leading genetic cause of infant deaths and about 9,000 people in the U.S. live with the condition.

Unlike Zolgensma, which is administered intravenously based on patient weight, Itvisma is a concentrated formulation administered directly to the central nervous system through the spinal cord. The new treatment does not need to be adjusted for the patient's weight, the company said.

Both therapies replace the SMN1 gene, offering the potential to reduce the need for chronically administered treatment associated with other available therapies for this population.

Zolgensma generated $925 million in global sales in the first nine months of 2025.

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