BioSpectrum
the business of Bio & Health Sciences
ASIA EDITION

US FDA approves Novartis' gene therapy for treatment of spinal muscular atrophy

28 November 2025 | News

Itvisma is an adeno-associated virus (AAV) vector-based gene therapy

The US Food and Drug Administration (FDA) has approved Itvisma (onasemnogene abeparvovec-brve) for the treatment of spinal muscular atrophy (SMA) in adult and paediatric patients 2 years of age and older with confirmed mutation in the survival motor neuron 1 (*SMN1*) gene. Itvisma is an adeno-associated virus (AAV) vector-based gene therapy.

SMA is an autosomal-recessive neurodegenerative disorder caused by mutations in the *SMN*1 gene, characterised by irreversible and progressive motor neuron loss, leading to progressive muscle atrophy and weakness, and subsequent paralysis and death in the most severe cases. SMA has an incidence of approximately 4-10 per 10,000 live births. Prior to the availability of effective treatment, SMA was considered one of the leading causes of infant mortality due to genetic disease in the US.

The direct administration of Itvisma into the cerebrospinal fluid surrounding the spinal cord (site of action) allows for delivery to motor neurons with a lower dose of vector, without the need to adjust for the patient's body weight. This provides a treatment with rapid onset and direct targeting of the genetic root cause of SMA. By addressing the root cause of SMA, Itvisma restores SMN protein production and halts further disease progression.

The FDA granted this application Fast Track, Breakthrough Therapy, and Priority Review designations. Itvisma also received Orphan Drug designation, which provides incentives to encourage the development of drugs for rare diseases. Itvisma is manufactured by Novartis Gene Therapies, Inc.