23 April 2026
EMADOC-1829012207-48071
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Itvisma

onasemnogene abeparvovec

On 23 April 2026, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Itvisma[2], intended for the treatment of spinal muscular atrophy (SMA). As Itvisma is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies.

The applicant for this medicinal product is Novartis Europharm Limited.

Itvisma will be available as a 1.2×10^{14} vector genomes solution for injection. The active substance of Itvisma is onasemnogene abeparvovec, a gene therapy (ATC code: M09AX09). Onasemnogene abeparvovec is a non-replicating recombinant viral vector designed to introduce a functional copy of the survival motor neuron gene (*SMN1*) in the transduced cells to address the monogenic root cause of SMA. By providing an alternative source of SMN protein expression in motor neurons, it is expected to promote the survival and function of transduced motor neurons.

The benefit of Itvisma is an improvement in motor function in children from 2 years of age with SMA who have bi-allelic mutation in the *SMN1* gene. The most common side effects with Itvisma include upper respiratory tract infection, pyrexia, vomiting, headache and increased hepatic enzymes.

The full indication is:

> Itvisma is indicated for the treatment of 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the *SMN1* gene in patients 2 years of age and older.

Treatment with Itvisma should be initiated and administered in clinical centres and supervised by a physician experienced in the management of patients with SMA.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.