EMA/126572/2026
EMEA/H/C/006498

Itvisma (*onasemnogene abeparvovec*)

A plain-language overview of Itvisma and why it is authorised in the EU

What is Itvisma and what is it used for?

Itvisma is a gene therapy medicine for treating spinal muscular atrophy (SMA), a serious condition of the nerve cells (neurons) that causes muscle wasting and weakness, in people from 2 years of age with inherited mutations (changes) in the SMN1 gene.

SMA is rare, and Itvisma was designated an 'orphan medicine' (a medicine used in rare diseases) on 19 June 2015. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3151509.

Itvisma contains the active substance onasemnogene abeparvovec.

How is Itvisma used?

Itvisma can only be obtained with a prescription, and treatment should be started, given and supervised by a doctor with experience in the treatment of SMA.

The medicine is available as a solution for injection. It is given only once by intrathecal injection (in the lower back, directly into the fluid around the spinal cord) in a hospital setting by a doctor experienced in carrying out this procedure. Patients may need to be sedated (given a medicine to calm them) before they are given Itvisma.

Patients will also be given prednisolone (or another corticosteroid) by mouth, 24 hours before receiving Itvisma. Daily corticosteroid treatment will last for about 2 months after the dose of Itvisma, or until the patient's liver enzyme levels decrease to an acceptable level. The doctor will slowly reduce the dose of corticosteroid until it can be fully stopped.

For more information about using Itvisma, see the package leaflet or contact your doctor or pharmacist.

How does Itvisma work?

Patients with SMA have a defect in a gene called SMN1, which the body needs to make a protein essential for the survival and normal functioning of lower motor neurons, the nerve cells from the spinal cord that control muscular movements. The active substance in Itvisma, onasemnogene abeparvovec, contains a functional copy of this gene. When injected, it passes into the motor neurons

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

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from where it provides the correct gene to make enough of the protein and thereby improve the motor function.

What benefits of Itvisma have been shown in studies?

A main study involving 126 children with SMA aged 2 to less than 18 years, who had not been treated before with other SMA medications and who were able to sit but have never been able to walk independently, showed that Itvisma is effective at improving motor function when compared with placebo (a sham injection).

The main measure of effectiveness was the change in motor function assessed with a scale called HFMSE (Hammersmith functional motor scale – expanded) 1 year after receiving Itvisma. The change in HFMSE score was 2.4 in patients receiving Itvisma, compared with 0.5 in those given placebo. This indicates an improvement of the motor abilities, like changing posture or moving their arms, in the treated patients after a year.

Studies carried out with Itvisma are described in more detail in the medicine's assessment report.

What are the side effects and restrictions with Itvisma?

For the full list of side effects and restrictions with Itvisma, see the package leaflet.

The most common side effects with Itvisma (which may affect more than 1 in 10 people) include upper respiratory tract infection (nose and throat infection), fever, vomiting and headache. Other common side effects (which may affect up to 1 in 10 people) are increased levels of liver enzymes.

Some side effects can be serious. The most frequent (which may affect up to 1 in 10 people) include vomiting, increased levels of liver enzymes, headache and fever.

Why is Itvisma authorised in the EU?

Itvisma has been shown to lead to clinically meaningful improvements in motor function in patients aged 2 years and older (children, adolescents and adults) with SMA. Side effects were similar to those with another medicine containing the same active substance and were considered manageable. The European Medicines Agency therefore decided that Itvisma's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Itvisma?

The company that markets Itvisma will provide educational materials to healthcare professionals, patients and caregivers with information on SMA, how to use the medicine safely, the risks associated with the medicine and how to identify and report side effects.

These materials may be made available by national competent authorities on their websites. A list of national repositories is available on the EMA website.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Itvisma have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Itvisma are continuously monitored. Suspected side effects reported with Itvisma are carefully evaluated and any necessary action taken to protect patients.

Other information about Itvisma

Itvisma received a marketing authorisation valid throughout the EU on 30 June 2026.

Further information on Itvisma, including the package leaflet and assessment report, can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/itvisma.

For information about the availability of this medicine in your country, contact your national competent authority.

This overview was last updated in 07-2026.