



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Spinraza (*nusinersen*)

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

What is Spinraza and what is it used for?

Spinraza is a medicine used to treat 5q spinal muscular atrophy (SMA), a genetic disease that causes weakness and wasting of the muscles including the lung muscles. The disease is linked to a defect on chromosome 5q and symptoms usually start shortly after birth.

SMA is rare, and Spinraza was designated an 'orphan medicine' (a medicine used in rare diseases) on 2 April 2012. Further information on the orphan designation can be found on the [EMA website](#).

Spinraza contains the active substance nusinersen.

How is Spinraza used?

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

The medicine is available as a solution for injection. It is given by intrathecal injection (into the lower back, directly into the spine) by a doctor or nurse experienced in carrying out this procedure. The patient may need to be sedated (given a medicine to calm them) before they are given Spinraza.

Spinraza should be given as soon as possible after the patient has been diagnosed with SMA. Treatment should be continued for as long as the patient benefits from it.

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

How does Spinraza work?

Patients with SMA lack a protein called 'survival motor neuron' (SMN) protein, which is essential for motor neurons (nerve cells from the spinal cord that control muscle movements) to survive and

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function normally. The SMN protein is made from two genes, SMN1 and SMN2. Patients with SMA lack the SMN1 gene but have the SMN2 gene, which mostly produces a short SMN protein that does not work as well as a full-length protein.

Spinraza is a synthetic anti-sense oligonucleotide (a type of genetic material) that enables the SMN2 gene to produce full length protein, which is able to work normally. This replaces the missing protein, thereby relieving the symptoms of the disease.

What benefits of Spinraza have been shown in studies?

One main study, involving 121 babies (of an average age of 7 months) with SMA, showed that Spinraza is effective in improving movement when compared with placebo (sham injection).

After one year of treatment, 51% of babies receiving Spinraza (37 out of 73) showed progress in developing head control, rolling, sitting, crawling, standing and walking, whereas no similar progress was seen in any of the babies who received placebo. In addition, most babies treated with Spinraza survived for longer and needed breathing support later than those given placebo.

Another study assessed Spinraza's effectiveness in children whose SMA was less severe and diagnosed at a later stage (average age of 3 years). After 15 months of treatment, 57% of children receiving Spinraza showed improvement in movement compared with 26% of children on placebo.

Studies carried out with Spinraza are described in more detail in the medicine's assessment reports.

What are the side effects and restrictions with Spinraza?

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

The most common side effects with Spinraza (which may affect more than 1 in 10 people) include headache, back pain and vomiting. These side effects are thought to be caused by the injections into the spine used to give the medicine. In babies some side effects could not be assessed, as they could not communicate them.

Why is Spinraza authorised in the EU?

In its assessment, the European Medicines Agency recognised the serious nature of the disease and the urgent need for effective treatments.

Spinraza has been shown to lead to clinically meaningful improvements in young children with varying degrees of disease severity. Although the medicine was not tested in patients with the most severe and the mildest forms of SMA, it is expected to provide similar benefits to these patients.

Side effects were considered manageable, with most side effects related to the way the medicine is given.

The Agency therefore decided that Spinraza's benefits are greater than its risks and recommended that it be approved for use in the EU.

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

The company that markets Spinraza will complete and submit the results of an ongoing study on the long-term safety and effectiveness of the medicine in patients who are not yet showing symptoms of SMA.

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

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

Other information about Spinraza

Spinraza received a marketing authorisation valid throughout the EU on 30 May 2017.

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

For information about the availability of this medicine in your country, contact your [national competent authority](#).

This overview was last updated in 03-2026.