



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Evrysdi (*risdiplam*)

An overview of Evrysdi and why it is authorised in the EU

What is Evrysdi and what is it used for?

Evrysdi is a medicine used to treat patients with 5q spinal muscular atrophy (SMA), a genetic disease that causes weakness and wasting of the muscles including the lung muscles. It is intended for patients with SMA type 1, type 2 or type 3, or those who have up to 4 copies of a gene known as *SMN2*.

SMA is rare, and Evrysdi was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 February 2019. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3192145.

Evrysdi contains the active substance risdiplam.

How is Evrysdi used?

Treatment with Evrysdi must be started by a doctor experienced in treating SMA. The medicine can only be obtained with a prescription.

Evrysdi is taken by mouth once a day after a meal, at around the same time each day. In patients who are not able to swallow, Evrysdi can be given via a tube through the nose or skin to the stomach.

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

How does Evrysdi work?

Patients with SMA lack a protein called 'survival motor neuron' (SMN) protein, which is essential for motor neurons (nerve cells in the spinal cord that control muscle movement) to continue working normally. Two genes, *SMN1* and *SMN2*, are involved in the production of the SMN protein. Patients with SMA lack the *SMN1* gene but have one or more copies of the *SMN2* gene, which mostly produce a short SMN protein that does not work as well as the full-length protein.

The active substance in Evrysdi, risdiplam, is a small molecule that allows the *SMN2* gene to produce the full-length protein, which can work normally. This is expected to increase survival of motor neurons, thereby reducing current symptoms of the disease and slowing down its progression.

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What benefits of Evrysdi have been shown in studies?

Evrysdi was shown to be effective at improving motor function in 2 main studies in patients with SMA.

One study conducted in 41 infants aged 2 to 7 months with type 1 SMA (the most severe type) showed that 29% (12 out of 41) of infants were able to sit without support for more than 5 seconds after 12 months of treatment with Evrysdi. Past observations of infants with SMA found that they are never able to sit without support.

A second study in 180 patients with type 2 and type 3 SMA up to 25 years of age showed a slight improvement in motor function (as measured using a rating scale called MFM32) in patients treated with Evrysdi: there was a 1.6-point difference compared with placebo (a dummy treatment) on a 100-point scale after 12 months of treatment.

Data from an additional study involving 18 newborn infants up to 6 weeks of age at the start of treatment support the use of Evrysdi in infants diagnosed with SMA but not yet showing symptoms. Of the seven children who received Evrysdi for at least 12 months, six reached milestones (such as sitting without support) which could normally not be attained by untreated children with 2 copies of *SMN2*.

What are the risks associated with Evrysdi?

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

The most common side effects with Evrysdi (which may affect more than 1 in 10 people) include fever, rash, diarrhoea and headache.

Why is Evrysdi authorised in the EU?

The effects of Evrysdi on the development of motor function in patients with SMA type 1, 2 and 3 were considered relevant, particularly considering the severity of the disease. In children with type 1 SMA, the most severe form of the disease, Evrysdi allows infants to sit without support for more than 5 seconds after a year of treatment, which they would not be able to do without treatment.

Evrysdi is also beneficial in patients with a later onset of SMA (type 2 and 3), although the effects in these patients are modest. The side effects with Evrysdi are considered manageable. Therefore, the European Medicines Agency decided that Evrysdi'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 Evrysdi?

The company that markets Evrysdi will provide data from a long-term study on the effects of the medicine in patients with up to 4 copies of the *SMN2* gene compared with how the disease progresses in patients who have not been treated with Evrysdi.

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

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

Other information about Evrysdi

Evrysdi received a marketing authorisation valid throughout the EU on 26 March 2021.

Further information on Evrysdi can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/evrysdi.

This overview was last updated in 08-2023.