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Evrysdi® (active substance: risdiplam)

First authorisation in Switzerland: 6 May 2021

Medicinal product (powder for oral solution) for the treatment of spinal muscular atrophy

About the medicinal product

Evrysdi, containing the active substance risdiplam, is used for the treatment of so-called 5q-associated spinal muscular atrophy (SMA) in patients 2 months of age and older.

Spinal muscular atrophy is a genetic disease that can be present at birth. SMA is caused by a deficiency in the body of a protein called "survival motor neuron" (SMN). A shortage of SMN protein can cause a loss of motor nerve cells, leading to muscle weakness and muscle wasting. Basic activities such as head and neck control, sitting, crawling and walking can be affected as a result. The muscles used for breathing and swallowing may also be affected.

Spinal muscular atrophy is classified in severities ranging from type I to type IV. Evrysdi is used for the treatment of SMA types I, II and III.

Since this is a rare disease, the medicine has been authorised as an orphan drug. The term "orphan drug" refers to important medicines for rare diseases that meet specific requirements.

Mode of action

Evrysdi works by helping the body produce more of the required SMN protein. As a result, fewer nerve cells are lost in patients of various ages and with different forms of SMA, potentially improving the strength and function of the muscles.

Use

Evrysdi is available only on prescription. Before it is dispensed, the Evrysdi oral solution must be reconstituted from powder by a healthcare professional, such as a doctor or pharmacist.

Evrysdi can be taken/administered either by mouth or via a nasogastric tube. Before the first dose is taken/administered, a professional should provide detailed instructions on how the daily dose must be prepared and taken/administered. Evrysdi should be taken/administered once daily after a meal, at around the same time each day.

The doctor determines the appropriate daily dose of Evrysdi in children aged 2 months and older based on the age and weight of the child. For adolescents and adults, the daily dose of Evrysdi is 5 mg (6.6 ml of oral solution). The re-usable syringes provided in the carton should be used to measure the dose.

The safety and efficacy of Evrysdi in children under 2 months have not yet been established.

Efficacy

The efficacy of Evrysdi in the treatment of patients with SMA since birth and SMA with later onset was investigated in the two main studies FIREFISH and SUNFISH. Both studies showed that Evrysdi improves motor function.

In the FIREFISH study, 41 patients aged from 2 to 7 months with type I SMA were treated with Evrysdi. SMA type I is the severest form of spinal muscular atrophy. The study investigated how many patients were able to sit without support for at least 5 seconds after 12 months of treatment with Evrysdi. This ability was demonstrated in 29% (12 out of 41) of the patients. Previous experience shows that untreated infants with SMA type I were never able to sit without support.
In the SUNFISH study, 180 patients with type II (71%) or type III (29%) SMA aged from 2 to 25 years were treated with Evrysdi or placebo (dummy drug). An improvement in motor function was achieved in those patients who were treated with Evrysdi. This improvement was measured on a 100-point rating scale called MFM32. After a 12-month treatment with Evrysdi, a difference of 1.6 points compared to the treatment with placebo was shown. An improvement in the MFM32 total score of 3 or more points was observed in 38% of the patients treated with Evrysdi and 24% of the patients treated with placebo.

Efficacy over treatment periods of up to two years was demonstrated in the studies. Limited data are available for periods longer than two years.

Precautions, undesirable effects & risks

Evrysdi must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects of Evrysdi are diarrhoea, rash and fever.

All precautions, risks and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicine has been authorised

In infants with SMA, Evrysdi can improve the chances of survival and preserve the ability to take food by mouth. Evrysdi can also increase the likelihood of achieving important development steps, known as "motor milestones", and reduce the need for a ventilator to help with breathing. In children (from infants to adolescents) and adults, Evrysdi can maintain or improve motor function over time.

Based on all the available data, the benefits of Evrysdi outweigh the risks. Swissmedic has therefore authorised the medicinal product Evrysdi, containing the active substance risdiplam, for use in Switzerland for patients aged 2 months and older with 5q-associated SMA.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Evrysdi®

Information for patients (package leaflet): Information for patients Evrysdi®

Healthcare professionals (doctors, pharmacists and others) can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

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