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# Evrysdi® (active substance: risdiplam)

## Indication extension in Switzerland: 12 December 2022

## 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 5q-associated spinal muscular atrophy (SMA) in patients.

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 1 to type 4. Evrysdi is used for the treatment of SMA types 1, 2 and 3.

Swissmedic approved the medicinal product Evrysdi for the treatment of 5q-associated spinal muscular atrophy (SMA) in patients 2 months of age and older, on 6 May 2021. The present indication extension means that patients under the age of 2 months with genetically diagnosed SMA can now also be treated with Evrysdi.

Since this is a rare and life-threatening disease, the medicine has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

## Mode of action

Evrysdi helps the body produce more of the required SMN protein. As a result, fewer nerve cells are lost, 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 is taken/administered once daily after a meal.

Evrysdi should be taken/administered at approximately the same time each day.

The doctor determines the appropriate daily dose of Evrysdi in children based on the age and weight of the child. The re-usable syringes provided in the carton should be used to measure the dose.

The safety and efficacy of Evrysdi in infants aged under 16 days have not yet been established.

## Efficacy

The interim results of an ongoing study (RAINBOWFISH) were taken into account for the present indication extension for the treatment of infants aged under 2 months with Evrysdi. In this study, treatment with Evrysdi was initiated in children from birth to 6 weeks of age. SMA had been genetically diagnosed in the infants, without their having already shown symptoms of the disease.

The currently available efficacy data related to 7 infants aged between 16 and 40 days who were treated for at least 12 months. They achieved important milestones in bodily function during the monitoring period. For example, all 7 infants were able to sit unaided. In untreated infants with SMA, this ability is usually lost rapidly.

## 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 side effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

## Why the medicinal product has been authorised

Despite the limited clinical data in regard to the number of patients investigated and the duration of treatment, the benefit-risk relationship is considered to be positive for the requested indication.

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 under 2 months with SMA.
As a condition of the authorisation, the final study data from RAINBOWFISH must be submitted.

## 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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