Healthcare Improvement Scotland | SMC Advice on new medicines

www.scottishmedicines.org.uk

Decision Explained

Medicine: risdiplam (brand name: Evrysdi®)

Roche Products Limited

The Scottish Medicines Consortium (SMC) has assessed risdiplam to treat 5q spinal muscular atrophy (SMA) in patients who are 2 months or older with a diagnosis of type 1, type 2 or type 3 SMA, or who have between one and four copies of a gene called SMN2. This document summarises the SMC decision and what it means for patients.

What has SMC said?

After careful consideration, SMC has accepted risdiplam for the treatment of SMA, as described above.

This SMC advice takes into account a confidential discount offered by the pharmaceutical company that improves the cost-effectiveness of risdiplam. In addition, SMC was able to apply a more flexible approach* in the assessment, as it is for a rare condition.

What does SMC's decision mean for patients?

If your healthcare professional thinks that risdiplam for use as described above is the right medicine for you or your child, you or they should be able to have the treatment on the NHS in Scotland.

What is risdiplam used for?

Risdiplam is used for the treatment of 5q spinal muscular atrophy (SMA). SMA is a rare muscle wasting disease caused by a mutation (a fault) in a gene called SMN1 which is located on chromosome 5q. This gene is needed to make a protein called the survival motor neuron (SMN) protein, which is critical for the function of the nerves that control muscles. There is a lot of variation in the severity of symptoms experienced by patients with different types of SMA. Increasing severity is associated with being younger when the symptoms first appear and in patients that have fewer SMN2 gene copies. Risdiplam is used for treating patients with SMA who are 2 months or older with type 1, type 2 or type 3 SMA, or who have between one and four copies of the SMN2 gene.

How does risdiplam work?

Patients with SMA do not produce enough of the SMN protein. This protein helps the nerve cells that control muscle movement (motor neurons) to survive and work properly. There are two genes that produce the SMN protein: SMN1 and SMN2. People with SMA do not have an SMN1 gene that works properly. They still have one or more copies of the SMN2 gene which can produce an SMN protein

*https://www.scottishmedicines.org.uk/how-we-decide/pace/

but the protein is shortened and it doesn't function well. Risdiplam works by letting the SMN2 gene produce a full-length SMN protein that helps the motor neurons to survive and work properly. This can help slow the progression of the disease.

How does SMC make its decision?

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland.

To do this SMC considers the following:

- Evidence from the company about how well the medicine works compared with current treatments available in Scotland, in relation to how much they will cost to buy and administer.
- Information from patient groups about the potential impact of the medicine on patients and carers.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.

When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not only those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of risdiplam by looking at the SMC Detailed Advice Document (SMC2401).

More information

The organisations below can provide more information and support for people with SMA and their families. SMC is not responsible for the content of any information provided by external organisations.

SMA UK

https://smauk.org.uk 01789 267520

Muscular Dystrophy UK

https://www.musculardystrophyuk.org 0800 652 6352

TreatSMA

https://www.treatsma.uk 0300 800 0202

You can find out more about risdiplam (Evrysdi®) in the Patient Leaflet by searching for the medicine name on the electronic medicines compendium (EMC) website.

https://www.medicines.org.uk/emc/