

Risdiplam for treating spinal muscular atrophy

Information for the public

Published: 16 December 2021

Last updated: 15 December 2023

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Risdiplam (Evrysdi) is available on the NHS as a possible treatment for 5q spinal muscular atrophy (SMA) in people of all ages.

Managed access period

You can only have it through something called a [managed access agreement](#). A managed access agreement is used when NICE thinks a treatment is promising but is not sure about its long-term benefits, and wants to collect more evidence before it makes a final decision.

To have risdiplam, you need to have either:

- a diagnosis of SMA types 1, 2 or 3, or
- confirmed SMA before you have any symptoms, and 1 to 4 copies of a gene called SMN2.

You'll also be asked if you're happy for your data to be collected so that the NHS can understand how patients benefit from risdiplam. This includes things like the number of chest infections you've had, any problems with swallowing, and how well you think the treatment is working.

What happens at the end of the managed access period

Evidence is likely to be collected on risdiplam until March 2024. After this NICE will review the new evidence collected during the managed access period, decide whether or not to recommend it for routine use in the NHS, and update the guidance.

If the evidence shows that risdiplam does not work well and is not good value for the NHS, it will no longer be recommended. But you will be able to continue receiving it for as long as you and your clinicians agree it is helping you.

Is this treatment right for me?

Your healthcare professionals should give you clear information, talk with you about your options and listen carefully to your views and concerns. Your family can be involved too, if you wish. Read more about [making decisions about your care](#).

Questions to think about

- Why do you need to collect my data?
- What extra appointments will I need?
- How well does it work compared with other treatments?
- What are the risks or side effects? How likely are they?
- What if I'm already having treatment?
- How will the treatment affect my day-to-day life?
- What happens if the treatment does not work?
- What happens if I do not want to have treatment? Are there other treatments available?
- What will happen to my treatment if NICE no longer recommends risdiplam?

Information and support

The [NHS website](#) may be a good place to find out more.

These organisations can give you advice and support:

- [Spinal Muscular Atrophy UK](#), 01789 267520
- [Treat SMA](#), 0300 800 0202
- [Muscular Dystrophy UK](#), 0800 652 6352

You can also get support from your local [Healthwatch](#).

NICE is not responsible for the quality or accuracy of any information or advice provided by these organisations.

ISBN: 978-1-4731-5651-7