

Onasemnogene abeparvovec for treating spinal muscular atrophy

Information for the public

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Onasemnogene abeparvovec (Zolgensma) is available on the NHS as a possible treatment for 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of type 1 SMA in babies, only if:

- they are 6 months or younger, or
- they are aged 7 to 12 months, and their treatment is agreed by the national multidisciplinary team.

It is only recommended for these groups if neither tracheostomy nor permanent ventilation for more than 16 hours per day is needed. For babies aged 7 to 12 months, it will be offered if treatment will give them at least a 70% chance of being able to sit independently.

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

- How well does it work compared with other treatments?
- What are the risks or side effects? How likely are they?
- 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?

Information and support

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

These organisations can give you advice and support:

- [Muscular Dystrophy UK](#), 0800 652 6352
- [Spinal Muscular Atrophy UK](#), 01789 267520
- [Treat SMA](#), 0300 800 0202
- [The Annabelle Rose Foundation for Spinal Muscular Atrophy](#), 0300 8000 762

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.

Update information

April 2023: Recommendation 1.3 in [NICE's highly specialised technologies guidance on onasemnogene abeparvovec for treating spinal muscular atrophy](#) was updated and replaced by [NICE's highly specialised technologies guidance on onasemnogene abeparvovec for treating presymptomatic spinal muscular atrophy](#). So, the information on that recommendation has been deleted from this information for the public.

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