

Onasemnogene abeparvovec for treating presymptomatic spinal muscular atrophy

Information for the public Published: 19 April 2023

www.nice.org.uk

Onasemnogene abeparvovec (Zolgensma) is available on the NHS as a possible treatment for presymptomatic 5q spinal muscular atrophy with a biallelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene in babies aged 12 months and under.

Is this treatment right for my baby?

Your healthcare professionals should give you clear information, talk with you about your baby's options, and listen carefully to your views and concerns. Your family can be involved too, if you wish. See <u>our webpage on making decisions about your care</u>.

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 baby's day-to-day life?
- What happens if the treatment does not work?
- What happens if I do not want my baby to have treatment? Are there other treatments available?

Information and support

The <u>NHS webpage on spinal muscular atrophy</u> may be a good place to find out more.

These organisations can give you advice and support:

- Spinal Muscular Atrophy UK, 01789 267520
- Muscular Dystrophy UK, 0207 803 4800

You can also get support from your local <u>Healthwatch</u>.

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

ISBN: 978-1-4731-5135-2