



Medicine: onasemnogene abeparvovec (brand name: Zolgensma®)

Novartis Gene Therapies

The Scottish Medicines Consortium (SMC) has assessed onasemnogene abeparvovec for the treatment of: patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the survival motor neuron 1 (SMN1) gene (where both copies of the SMN1 gene are faulty) and a clinical diagnosis of SMA type 1; or patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to three copies of the SMN2 gene. This document summarises the SMC decision and what it means for patients.

What has SMC said?

After careful consideration, SMC has accepted onasemnogene abeparvovec for use in certain patients (restricted use). The restriction means that onasemnogene abeparvovec can be used to treat patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA type 1. Under this restriction, it can also be used to treat pre-symptomatic patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene, where patients are expected to develop SMA type 1.

This SMC advice takes into account a confidential discount offered by the pharmaceutical company that improves the cost-effectiveness of onasemnogene abeparvovec. 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 onasemnogene abeparvovec 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 onasemnogene abeparvovec used for?

Onasemnogene abeparvovec is used for the treatment of 5q spinal muscular atrophy (SMA). SMA is a rare muscle wasting disease caused by a mutation (faulty copy) in a gene called SMN1. 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. Onasemnogene abeparvovec is for patients where both copies of the SMN1 gene are faulty and who have a clinical diagnosis of SMA type 1 (the most severe form of SMA). It may also be used in some affected patients before they develop symptoms of SMA. Patients who have SMA type 1 usually die before 2 years of age and it is one of the main genetic causes of death in infants

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

How does onasemnogene abeparvovec work?

Onasemnogene abeparvovec is a type of medicine called a gene therapy that is given as a single dose via a one off infusion into a vein. It contains a copy of the SMN1 gene that can help the nerves make enough of the SMN protein to help some patients survive for longer and function more independently. Onasemnogene abeparvovec offers a therapeutic advancement and has the potential to significantly improve the patient's quality of life. Earlier treatment may help maximise its benefits.

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 just those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of onasemnogene abeparvovec by looking at the SMC Detailed Advice Document (SMC2311).

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.

Spinal Muscular Atrophy UK



<https://smauk.org.uk>



01789 267520

Muscular Dystrophy UK



<https://www.musculardystrophyuk.org>



0800 6526352

TreatSMA



<https://www.treatsma.uk>



0300 800 0202

You can find out more about onasemnogene abeparvovec (Zolgensma®) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



<http://www.ema.europa.eu>