



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Zolgensma (*onasemnogene abeparvovec*)

An overview of Zolgensma and why it is authorised in the EU

What is Zolgensma and what is it used for?

Zolgensma is a gene therapy medicine for treating spinal muscular atrophy, a serious condition of the nerves that causes muscle wasting and weakness.

It is intended for patients with inherited mutations affecting genes known as SMN1, who have either been diagnosed with SMA type 1 (the most severe type) or have up to 3 copies of another gene known as SMN2.

Spinal muscular atrophy is rare, and Zolgensma was designated an 'orphan medicine' (a medicine used in rare diseases) on 19 June 2015. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3151509.

Zolgensma contains the active substance onasemnogene abeparvovec.

How is Zolgensma used?

Zolgensma is given once as an infusion (drip) into a vein lasting about 1 hour. The infusion should take place in a clinic or hospital under the supervision of a doctor experienced in managing spinal muscular atrophy.

Before and after receiving the infusion, the patient will have a number of tests, including liver and blood tests, and will be given corticosteroid medicines to reduce the risk of side effects. The medicine can only be obtained with a prescription. For more information about using Zolgensma, see the package leaflet or contact your doctor or pharmacist.

How does Zolgensma work?

Patients with spinal muscular atrophy have a defect in a gene known as *SMN1*, which the body needs to make a protein essential for the normal functioning of nerves that control muscle movements. The active substance in Zolgensma, onasemnogene abeparvovec, contains a functional copy of this gene. When injected, it passes into the nerves from where it provides the correct gene to make enough of the protein and thereby restore nerve function.

Official address Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands

Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us

Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

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What benefits of Zolgensma have been shown in studies?

A main study showed that Zolgensma reduces the need for artificial ventilation in babies with spinal muscular atrophy. In this study, 20 out of the 22 babies given Zolgensma were alive and breathing without a permanent ventilator after 14 months, when normally only a quarter of untreated patients would survive without needing a ventilator.

The study also showed that Zolgensma can help babies sit unaided for at least 30 seconds. 14 out of the 22 babies given Zolgensma were able to do so after 18 months, a milestone that is never achieved in untreated babies with severe forms of the disease.

What are the risks associated with Zolgensma?

The most common side effects with Zolgensma (which may affect more than 1 in 10 people) are raised liver enzymes and vomiting. For the full list of restrictions and side effects of Zolgensma, see the package leaflet.

Why is Zolgensma authorised in the EU?

The main study of Zolgensma showed that a one-time infusion can improve survival in these patients and reduce the need for a permanent ventilator to breathe. It can also help them reach development milestones.

As for its safety, the side effects of Zolgensma are considered manageable; the most common side effect in the study, raised liver enzymes, resolved after treatment with a steroid.

The European Medicines Agency therefore decided that Zolgensma's benefits are greater than its risks and it can be authorised for use in the EU.

Zolgensma has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Zolgensma?

Since Zolgensma has been given conditional authorisation, the company that markets Zolgensma will provide additional data on its benefits and risks. These include data from 2 studies in patients younger than 6 months with SMA type I and one study in patients younger than 6 weeks who do not have symptoms but for whom the diagnosis of SMA is confirmed based on genetic testing.

What measures are being taken to ensure the safe and effective use of Zolgensma?

The company that markets Zolgensma will conduct a long-term study of the medicine's safety and effectiveness and provide data from another study to improve the consistency of the batches of the medicines.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Zolgensma have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Zolgensma are continuously monitored. Side effects reported with Zolgensma are carefully evaluated and any necessary actions taken to protect patients.

Other information about Zolgensma

Zolgensma received a conditional marketing authorisation valid throughout the EU on 18 May 2020.

Further information on Zolgensma can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/zolgensma

This overview was last updated in 05-2020.