

Zolgensma[®] 2×10^{13} vector genomes/mL solution for infusion
onasemnogene abeparvovec

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects your child may get. See the end of section 4 for how to report side effects.

Read all of this leaflet carefully before your child is given this medicine because it contains important information.

- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your child's doctor or nurse.
- If your child gets any side effects, talk to your child's doctor or nurse. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

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1. What Zolgensma is and what it is used for

What Zolgensma is

Zolgensma is a type of medicine called a 'gene therapy'. It contains the active substance onasemnogene abeparvovec, which contains human genetic material.

What Zolgensma is used for

Zolgensma is used to treat spinal muscular atrophy (SMA), a rare, serious inherited disease.

How Zolgensma works

SMA occurs when there is a missing or abnormal version of a gene needed to make an essential protein called 'Survival Motor Neuron' (SMN) protein. Lack of SMN protein causes nerves that control muscles (motor neurons) to die. This results in muscles becoming weak and wasting away, with eventual loss of movement.

This medicine works by supplying a fully functioning copy of the SMN gene which then helps the body produce enough SMN protein. The gene is delivered into the cells where it is needed using a modified virus that does not cause disease in humans.

2. What you need to know before your child is given Zolgensma

Do NOT use Zolgensma

- if your child is allergic to onasemnogene abeparvovec or any of the other ingredients of this medicine (listed in section 6).

Warnings and precautions

Your child's doctor will check for antibodies before treatment to help decide if this medicine is suitable for your child.

Infusion-related reactions and serious allergic reactions

Infusion-related side effects and serious allergic reactions can occur during, and/or shortly after, your child is given Zolgensma. Possible signs you need to look out for include itchy rash, pale skin, vomiting, swelling of face, lips, mouth or throat (which may cause difficulty in swallowing or breathing) and/or changes in heart rate and blood pressure. Tell your child's doctor or nurse immediately if you notice your child develops these or any other new signs or symptoms during, and/or shortly after, Zolgensma treatment. Before your child is discharged, the doctor will provide you with information on what to do in case your child experiences new side effects or side effects which come back once you leave the medical facility.

Liver problems

Talk to your child's doctor or nurse before this medicine is given if your child has had any liver problems. This medicine can lead to an increase in enzymes (proteins found within the body) produced by the liver or injury to the liver. Injury to the liver can lead to serious outcomes, including liver failure and death. Possible signs you need to look out for after your child is given this medicine include vomiting, jaundice (yellowing of the skin or of the whites of the eyes), or reduced alertness (see section 4 for more information). Tell your child's doctor straightaway if you notice your child develops any symptoms suggestive of injury to the liver.

Your child will have a blood test to check how well the liver is working before starting treatment with Zolgensma. Your child will also have regular blood tests for at least 3 months after treatment to monitor for increases in liver enzymes.

Infection

An infection (e.g. cold, flu or bronchiolitis) before or after Zolgensma treatment may lead to more serious complications. Caregivers and close contacts with the patient should follow infection prevention practices (e.g. hand hygiene, coughing/sneezing etiquette, limiting potential contacts). You need to look out for signs of an infection such as coughing, wheezing, sneezing, runny nose, sore throat or fever. Tell your child's doctor straightaway if you notice your child develops any symptoms suggestive of infection **before** or **after** Zolgensma treatment.

Regular blood tests

This medicine can lower blood-platelet counts (thrombocytopenia). You need to look out for possible signs of a low blood-platelet count after your child is given Zolgensma such as abnormal bruising or bleeding (see section 4 for more information). Most of the reported cases of a low blood-platelet count occurred within the first three weeks after the child was given Zolgensma.

Before starting treatment with Zolgensma, your child will have a blood test to check the amount of blood cells (including red blood cells and platelets), as well as troponin-I level in their body. They will also have a blood test to check their creatinine level, which is an indicator of how the kidneys are working. Your child will also have regular blood tests for a period of time after treatment to monitor for changes in platelet levels.

Increased levels of troponin-I (a heart protein)

Zolgensma can raise levels of a heart protein called troponin-I. This can be shown in laboratory tests that your child's doctor will perform as needed.

Abnormal clotting of blood in small blood vessels (thrombotic microangiopathy)

There have been reports of patients developing thrombotic microangiopathy generally within the first two weeks after Zolgensma treatment. Thrombotic microangiopathy is accompanied by a decrease in red blood cells and cells involved in clotting (platelets) and can be fatal. These blood clots could affect your child's kidneys. Your child's doctor may want to check your child's blood (platelet counts) and

blood pressure. Possible signs you need to look out for after your child is given Zolgensma include bruising easily, seizures (fits) or decrease in urine output (see section 4 for more information). Seek urgent medical attention if your child develops any of these signs.

Blood, organ, tissue and cell donation

After your child has been treated with Zolgensma, they will not be able to donate blood, organs, tissues or cells. This is because Zolgensma is a gene therapy medicine.

Other medicines and Zolgensma

Tell your child's doctor or nurse if your child is taking, has recently taken or might take any other medicines.

Prednisolone

Your child will also be given a corticosteroid medicine such as prednisolone for about 2 months or longer (see also section 3) as part of Zolgensma treatment. The corticosteroid medicine will help manage any increase in liver enzymes that your child could develop after being given Zolgensma.

Vaccinations

As corticosteroids can affect the body's immune (defence) system, **your child's doctor may decide to delay giving some vaccinations** while your child is receiving corticosteroid treatment. Talk to your child's doctor or nurse if you have any questions.

Zolgensma contains sodium

This medicine contains 4.6 mg sodium per mL, equivalent to 0.23% of the WHO recommended maximum daily intake of 2 g sodium for an adult. Each 5.5-mL vial contains 25.3 mg sodium, and each 8.3-mL vial contains 38.2 mg sodium.

Additional information for parents/caregiver

Advanced SMA

Zolgensma can rescue living motor neurons but does not rescue dead motor neurons. Children with less severe symptoms of SMA (such as absent reflexes or reduced muscle tone) may have sufficient living motor neurons to benefit significantly from Zolgensma treatment. Zolgensma may not work as well in children with severe muscle weakness or paralysis, breathing problems or who are not able to swallow, or in children who have significant malformations (such as heart defects), including patients with SMA Type 0, as there may be limited potential improvement after treatment with Zolgensma. Your child's doctor will decide if your child should be given this medicine.

Risk of tumours associated with potential insertion into the DNA

There is a possibility that therapies such as Zolgensma can insert into the DNA of human body cells. As a consequence, Zolgensma could contribute to a risk of tumours because of the nature of the medicine. You should discuss this with your child's doctor. In the event of a tumour, your child's doctor may take a sample for further evaluation.

Hygiene care

The active substance in Zolgensma may temporarily be excreted through your child's bodily waste; this is called 'shedding'. Parents and caregivers should follow good hand-hygiene for up to 1 month after your child is given Zolgensma. Wear protective gloves when coming into direct contact with your child's bodily fluids or waste and wash hands thoroughly afterwards with soap and warm running water, or an alcohol-based hand sanitiser. Double bags should be used to dispose of soiled nappies and other waste. Disposable nappies may still be disposed of in household waste.

You should continue to follow these instructions for at least 1 month after your child's treatment with Zolgensma. Talk to your child's doctor or nurse if you have any questions.

3. How Zolgensma is given

Zolgensma will be given by a doctor or nurse trained in the management of your child's condition.

The doctor will work out the amount of Zolgensma your child will receive according to your child's weight. Zolgensma is given intravenously (into a vein) by a single infusion (drip) over about 1 hour.

Zolgensma will be given to your child ONCE only.

Your child will also be given prednisolone (or another corticosteroid) by mouth, starting 24 hours before being given Zolgensma. The dose of corticosteroid will also depend on your child's weight. Your child's doctor will work out the total dose to give.

Your child will be given corticosteroid treatment daily for about 2 months after the dose of Zolgensma, or until your child's liver enzymes decrease to an acceptable level. The doctor will slowly reduce the dose of corticosteroid until treatment can be fully stopped.

If you have any further questions, ask your child's doctor or nurse.

4. Possible side effects

Like all medicines, this medicine can have side effects, although not everybody gets them.

Seek urgent medical attention if your child develops any of the following serious side effects:

Common (may affect up to 1 in 10 people)

- bruising or bleeding for longer than usual if your child has been hurt - these may be signs of a low blood-platelet count.

Uncommon (may affect up to 1 in 100 people)

- vomiting, jaundice (yellowing of the skin or of the whites of the eyes) or reduced alertness - these may be signs of injury to the liver (including liver failure).
- bruising easily, seizures (fits), decrease in urine output – these may be signs of thrombotic microangiopathy.
- infusion-related reaction (see section 2, "Warnings and precautions").

Rare (may affect up to 1 in 1 000 people)

- serious allergic reactions (see section 2, "Warnings and precautions").

Talk to your child's doctor or nurse if your child develops any other side effects. These can include:

Very common (may affect more than 1 in 10 people)

- increases in liver enzymes seen in blood tests.

Common (may affect up to 1 in 10 people):

- vomiting
- fever.
- increases in troponin-I (a heart protein) seen in blood tests.

Reporting of side effects

If your child gets any side effects, talk to your child's doctor or nurse. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via the Yellow Card Scheme at: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. By reporting side effects, you can help provide more information on the safety of this medicine.

5. How to store Zolgensma

Keep this medicine out of the sight and reach of children.

The following information is for healthcare professionals who will prepare and give the medicine.

Do not use this medicine after the expiry date which is stated on the vial label and carton after EXP. The expiry date refers to the last day of that month.

Vials will be transported frozen (at or below -60°C).

Upon receipt vials should be refrigerated at 2°C to 8°C immediately, and in the original carton. Zolgensma therapy should be initiated within 14 days of receipt of vials.

This medicine contains genetically-modified organisms. Unused medicine or waste material must be disposed of in compliance with the local guidelines on handling of biological waste. As this medicine will be given by a doctor, the doctor is responsible for the correct disposal of the product. These measures will help protect the environment.

6. Contents of the pack and other information

What Zolgensma contains

- The active substance is onasemnogene abeparvovec. Each vial contains onasemnogene abeparvovec with a nominal concentration of 2×10^{13} vector genomes/mL.
- The other ingredients are tromethamine, magnesium chloride, sodium chloride, poloxamer 188, hydrochloric acid (for pH adjustment) and water for injections.

What Zolgensma looks like and contents of the pack

Zolgensma is a clear to slightly opaque, colourless to faint white solution for infusion.

Zolgensma may be supplied in vials containing a nominal fill volume of either of 5.5 mL or 8.3 mL. Each vial is for single use only.

Each carton will contain between 2 to 14 vials.

Marketing Authorisation Holder

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For any information about this medicine, please contact the Marketing Authorisation Holder.

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The following information is intended for healthcare professionals only:

Important: Please refer to the Summary of Product Characteristics (SmPC) before using.

Each vial is for single use only.

This medicinal product contains genetically-modified organisms. Local guidelines on handling of biological waste should be followed.

Handling

- Zolgensma should be handled aseptically under sterile conditions.
- Personal protective equipment (including gloves, safety goggles, laboratory coat and sleeves) should be worn while handling or administering Zolgensma. Personnel should not work with Zolgensma if skin is cut or scratched.
- All spills of Zolgensma must be wiped with absorbent gauze pads and the spill area must be disinfected using a bleach solution followed by alcohol wipes. All clean-up materials must be double bagged and disposed of in accordance with local guidelines on handling of biological waste.
- All materials that may have come in contact with Zolgensma (e.g. vial, all materials used for injection, including sterile drapes and needles) must be disposed of in accordance with local guidelines on handling of biological waste.

Accidental exposure

Accidental exposure to Zolgensma must be avoided.

In case of accidental exposure to skin, the affected area must be thoroughly cleansed with soap and water for at least 15 minutes. In case of accidental exposure to eyes, the affected area must be thoroughly flushed with water for at least 15 minutes.

Storage

Vials will be transported frozen (at or below -60°C). Upon receipt vials should be refrigerated at 2°C to 8°C immediately, and in the original carton. Zolgensma therapy should be initiated within 14 days of receipt of vials. The date of receipt should be marked on the original carton before the product is stored in the refrigerator.

Preparation

Vials should be thawed before use:

- For packs containing up to 9 vials – thaw for approximately 12 hours in the refrigerator (2°C to 8°C) or 4 hours at room temperature (20°C to 25°C).
- For packs containing up to 14 vials – thaw for approximately 16 hours in the refrigerator (2°C to 8°C) or 6 hours at room temperature (20°C to 25°C).

Do not use Zolgensma unless thawed.

Once thawed, the medicinal product should not be re-frozen.

After thawing, gently swirl Zolgensma. Do NOT shake.

Do not use this medicine if you notice any particles or discoloration once the frozen product has thawed and prior to administration.

After thawing, Zolgensma should be given as soon as possible.

Administration

Zolgensma should be given to patients ONCE only.

The dose of Zolgensma and exact number of vials required for each patient is calculated according to the patient's weight (see SmPC sections 4.2 and 6.5).

To administer Zolgensma, draw the entire dose volume into the syringe. Once the dose volume is drawn into the syringe it must be administered within 8 hours. Remove any air in the syringe before administering to the patient via intravenous infusion through a venous catheter. Insertion of a secondary ('back-up') catheter is recommended in case of blockage in the primary catheter.

Zolgensma should be administered with the syringe pump as a single intravenous infusion with a slow infusion of approximately 60 minutes. It should be administered as an intravenous infusion only. It should not be administered as a rapid intravenous injection or bolus. Following completion of infusion, the line should be flushed with sodium chloride 9 mg/mL (0.9%) solution for injection.

Disposal

Any unused medicinal product or waste material should be disposed of in accordance with local guidelines on handling of biological waste.

Temporary Zolgensma shedding may occur, primarily through bodily waste. Caregivers and patient's families should be advised on the following instructions for the proper handling of patients' bodily fluids and waste:

- Good hand-hygiene (wearing protective gloves and washing hands thoroughly afterwards with soap and warm running water, or an alcohol-based hand sanitiser) is required when coming into direct contact with patient's bodily fluids and waste for a minimum of 1 month after Zolgensma treatment.
- Disposable nappies should be sealed in double plastics bags and can be disposed of in household waste.