# Zolgensma®

## Consumer Medicine Information (CMI) summary

The full CMI on the next page has more details. If you are worried about using this medicine, speak to your doctor or pharmacist.

▼ This medicine is new or being used differently. Please report side effects. See the full CMI for further details.

**WARNING: Important safety information is provided in a boxed warning in the full CMI. Read before your child is given this medicine.**

## 1. Why is my child being given Zolgensma?

Zolgensma contains onasemnogene abeparvovec. It is a gene therapy used to treat babies, toddlers and young children, who have a rare, serious inherited condition called "spinal muscular atrophy" (SMA).

For more information, see Section 1. Why is my child being given Zolgensma? in the full CMI.

## 2. What should I know before my child is given Zolgensma?

Do not use if your child is allergic to onasemnogene abeparvovec, or any of the ingredients listed at the end of this leaflet.

Talk to your doctor if your child has any other medical conditions.

For more information, see Section 2. What should I know before my child is given Zolgensma? in the full CMI.

## 3. What if my child is using other medicines?

Some medicines may interfere with Zolgensma and affect how it works.

A list of these medicines is in Section 3. What if my child is using other medicines? in the full CMI.

## 4. How will my child be given Zolgensma?

Children weighing between 2.6 and 21.0 kg only can be treated with Zolgensma.

Zolgensma is given to your child by a single infusion (drip). This will be given over a period of approximately 1 hour into a vein in your child's leg or arm. Before treatment your child will be given a medication called prednisolone or another corticosteroid. Your child will be given prednisolone/corticosteroid treatment daily for approximately 2 months after the dose of Zolgensma, or until your child's increased liver enzymes decrease to an acceptable level.

More instructions can be found in Section 4. How will my child be given Zolgensma? in the full CMI.

## 5. What should I know while my child is given Zolgensma?

| | |
|---|---|
| **Things you should do** | • Keep all your doctor's appointments after your child receives Zolgensma.<br>• Keep all the appointments for your child's blood tests so that your doctor can monitor your child's health after Zolgensma treatment. |
| **After your child is given Zolgensma** | • The active medicine in Zolgensma may pass for a short time through your child's bodily waste. Your child's doctor or nurse will explain to you the proper handling of your child's stools and disposable nappies.<br>• Continue to follow the handling instructions for at least 1 month after your child's treatment with Zolgensma. |

For more information, see Section 5. What should I know after my child is given Zolgensma? in the full CMI.

## 6. Are there any side effects?

There can be some serious side effects before and after Zolgensma treatment.

For more information, including what to do if you have any side effects, see Section 6. Are there any side effects? in the full CMI.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. You can report side effects to your doctor, or directly at www.tga.gov.au/reporting-problems.

**WARNING: HEPATOTOXICITY Acute serious liver injury, acute liver failure and increased liver enzymes can occur with ZOLGENSMA. Cases of acute liver failure with fatal outcomes have been reported. If your child has a pre-existing liver condition before Zolgensma they may be at increased risk. Your doctor will check the liver function of your child by tests. Corticosteroid medicine will be given before, and after, your child is given Zolgensma. Liver function will be checked for at least 3 months after treatment.**

# Zolgensma®

**Active ingredient(s):** *onasemnogene abeparvovec*

## Consumer Medicine Information (CMI)

This leaflet provides important information about using Zolgensma. **You should also speak to your doctor or pharmacist if you would like further information or if you have any concerns or questions about using Zolgensma.**

**Where to find information in this leaflet:**



## 1. Why is my child being given Zolgensma?

Zolgensma (also known as onasemnogene abeparvovec) is a type of medicine called a "gene therapy". It contains human genetic material.

Zolgensma is used to treat babies, toddlers and young children who have a rare, serious inherited condition called "spinal muscular atrophy" (SMA).

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. Zolgensma can rescue viable motor neurons, but does not rescue dead motor neurons.

### How does Zolgensma work?

Zolgensma 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 should I know before my child is given Zolgensma?

### Warnings

**Do not use Zolgensma if:**

- your child is allergic to onasemnogene abeparvovec, or any of the ingredients listed at the end of this leaflet.

**Your doctor will check if this medicine is suitable for your child before starting treatment. He/she will:**

- Test for antibodies which will help decide if this treatment is suitable.
- Check if your child has had any liver problems and check their liver function. Zolgensma can cause an immune response that could lead to an increase in enzymes produced by the liver or injury to the liver. Injury to the liver can lead to serious outcomes, including liver failure and death.
- Check how much your child's illness has advanced.
- Check your child for any signs of infection.
- Check platelets because they help blood to clot and they may get too low after taking Zolgensma.
- Check the amount of blood cells (including red blood cells and platelets) as well as creatinine level which is an indicator of how the kidneys are working.

**Infections**

If your child develops an infection (e.g. cold, flu or bronchiolitis) before or after being treated with Zolgensma this could possibly lead to more serious complications. Caregivers and close contacts with the patient should follow infection prevention practices (e.g., hand hygiene, respiratory/cough etiquette, limit potential contacts). Signs of a possible infection you need to look out for in your child include 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.

**Prednisolone**

Your child will also be given a medicine called prednisolone for a period of time as part of their treatment with Zolgensma. This is a type of medicine called a 'corticosteroid' which will help manage any potential increase in liver enzymes that your child could develop after being given Zolgensma. Your child's doctor will decide if your child should be given prednisolone or another corticosteroid.

**Vaccinations**

As corticosteroids can affect the body's immune system, your child's doctor may decide to delay giving any vaccinations to your child while he/she is receiving prednisolone/corticosteroid treatment. Talk to your child's doctor or nurse if you have any questions.

During treatment, your child may be at risk of developing certain side effects. It is important you understand these risks and how to monitor for them. See additional information under Section 6. Are there any side effects?

### Use In Children

- Zolgensma is suitable for use in babies, toddlers and children weighing between 2.6 and 21.0 kg, with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.
- The benefit of taking Zolgensma is reduced in children with severe muscle weakness, breathing problems, on a ventilator machine permanently, or who are not able to swallow. Your child's doctor will decide if your child should be given this medicine.

**Theoretical risk of tumours**

There is a possibility that medicines like Zolgensma can insert into the DNA of human body cells. As a result, Zolgensma could cause tumours because of the nature of the medicine.

## 3. What if my child is taking other medicines?

Tell your doctor or pharmacist if your child is taking any other medicines, including any medicines, vitamins or supplements that you buy without a prescription from your pharmacy, supermarket or health food shop.

**Some medicines may interfere with Zolgensma and affect how it works.**

Where possible, change your child's vaccination schedule to allow for prednisolone/corticosteroid use before and after Zolgensma infusion.

**Check with your doctor or pharmacist if you are not sure about what medicines, vaccines, vitamins or supplements, including medicines you may have bought from a health shop, you are giving your child and whether these may affect Zolgensma.**

## 4. How will my child be given Zolgensma?

### How much will be given

- Zolgensma will be given to your child ONCE only. The amount of medicine given is based on your child's weight. Your doctor will work out the correct dose to give.

### When will Zolgensma be given

- Children weighing between 2.6 and 21.0 kg only can be treated with Zolgensma.
- Your child will also be given treatment with prednisolone by mouth (or another corticosteroid), starting 24 hours before starting the Zolgensma infusion (drip). The dose of corticosteroid will also depend on your child's weight. The recommended dose of prednisolone is 1 mg per kg body weight daily. Your child's doctor will work out the total dose to give.

### How will Zolgensma be given

- Zolgensma will be given to your child by a doctor or nurse experienced in treating SMA
- Zolgensma is given to your child by a single infusion (drip). This will be given over a period of approximately 1 hour into a vein in your child's leg or arm.

## 5. What should I know after my child is given Zolgensma?

Your child will be given prednisolone (or another corticosteroid) daily for approximately 2 months after the dose of Zolgensma, or until your child's increased liver enzymes decrease to an acceptable level and all other assessments return to normal range. The dose of corticosteroid given to your child will be slowly decreased during this time until treatment can be fully stopped. Your child's doctor will explain when and how they will stop corticosteroid treatment for your child.

### Things you should do

Keep all your doctor's appointments and appointments for blood tests after your child receives Zolgensma.

The active medicine in Zolgensma may pass for a short time through your child's bodily waste. Your child's doctor or nurse will explain to you the proper handling of your child's stools and disposable nappies (e.g. using gloves when changing nappies, using bags to dispose of soiled nappies and other waste). Bagged disposable nappies can be disposed of in household waste.

They will also advise you on good hand-hygiene to use when coming into direct contact with your child's bodily 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.

If you have any further questions on the use of Zolgensma or prednisolone ask your child's doctor or nurse.

**After treatment your child will have regular blood tests:**

- Your child will have regular blood tests to monitor liver function for at least 3 months after treatment.

  Possible signs of liver problems include vomiting, jaundice (yellowing of the skin or of the whites of the eyes) or reduced alertness. Tell your child's doctor straight away if you notice your child develops any symptoms suggestive of injury to the liver.

- Zolgensma may lower the blood platelet count (thrombocytopenia). This has been observed to generally occur within the first two weeks after Zolgensma treatment. Your child will have regular blood tests to monitor for changes in blood platelets for up to 3 months after infusion.

  Possible signs of a low blood-platelet count you need to look out for after your child is given Zolgensma include abnormal bruising or bleeding.

**Call your doctor straight away if you notice:**

- A change in your child's skin colour to pale grey/blue skin colour or yellow skin colour.
- Increased tiredness (tires easily), or difficulty breathing (rapid breathing, shortness of breath) at rest or with activity in your child.
- Signs of an infection or sweats during feeding.
- Bruising easily, seizures (fits) or decrease in urine output. This has been observed to generally occur within the first two weeks after Zolgensma treatment.
- Bruising or bleeding for longer than usual if your child has been hurt.
- Signs of infusion-related effects such as rash, hives, vomiting, difficulty breathing, shortness of breath, changes in heart rate.

Remind any doctor, dentist, nurse or pharmacist you visit that your child was given Zolgensma.

## 6. Are there any side effects?

All medicines can have side effects. If your child experiences any side effects, most of them are minor and temporary. However, some side effects may need medical attention.

See the information below and, if you need to, ask your doctor or pharmacist if you have any further questions about side effects.

### Less serious side effects

| Less serious side effects | What to do |
|---|---|
| • Vomiting<br>• Fever | **Speak to your doctor if your child has any of these less serious side effects and they worry you.** |

### Serious side effects

| Serious side effects | What to do |
|---|---|
| • Bruising or bleeding for longer than usual if your child has been hurt - these may be signs of a low blood-platelet count.<br>• Pale grey or blue skin colour, difficulty in breathing (e.g. rapid breathing, shortness of breath), swelling of the legs/arms or tummy area - these may be signs of possible problems with the heart.<br>• Vomiting, yellowing of the skin and/or whites of the eyes or reduced alertness – these may be signs of injury to the liver including liver failure.<br>• Bruising easily, seizures (fits), decrease in amount of urine – these may be signs of thrombotic microangiopathy (TMA).<br>• Rash, hives, vomiting, difficulty breathing, shortness of breath, change in heart rate. | **Call your doctor straight away, or go straight to the Emergency Department at your nearest hospital if you notice any of these serious side effects.** |

**Tell your doctor or pharmacist if you notice anything else that may be making your child feel unwell.**

Other side effects not listed here may occur in some people.

Some side effects can only be found when your doctor does tests to check your child's progress. These include increase in liver enzymes (that give information about the health of the liver).

### Reporting side effects

After you have received medical advice for any side effects you experience, you can report side effects to the Therapeutic Goods Administration online at www.tga.gov.au/reporting-problems. By reporting side effects, you can help provide more information on the safety of this medicine.

## 7. Product details

Zolgensma is only given in a hospital.

### What Zolgensma contains

| | |
|---|---|
| **Active ingredient (main ingredient)** | Onasemnogene abeparvovec |

| | |
|---|---|
| **Other ingredients (inactive ingredients)** | Trometamol<br>Magnesium chloride hexahydrate<br>Sodium chloride<br>Poloxamer<br>Hydrochloric acid (for pH adjustment)<br>Water for injections |

**Do not use this medicine if your child is allergic to any of these ingredients.**

**Zolgensma contains less than 1 mmol sodium (23 mg) per mL sodium**

## What Zolgensma looks like

Zolgensma is supplied in a vial with a stopper, seal and coloured cap. The amount of liquid in each vial may be either 5.5 mL or 8.3 mL.

5.5 mL vial Aust R 327906

8.3 mL Aust R 327905

Combination pack Aust R 327907

## Who distributes Zolgensma

Zolgensma is supplied in Australia by:

Novartis Pharmaceuticals Australia Pty Limited

(ABN 18 004 244 160)

54 Waterloo Road

Macquarie Park NSW 2113

Telephone 1 800 671 203

Website: www.novartis.com.au

This leaflet was prepared in April 2026.

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Internal document code: (zol250326c_v2) based on PI (zol250326i)