Itvisma®

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.

1. Why is my child being given Itvisma?

Itvisma contains onasemnogene abeparvovec. It is a gene therapy used to treat children aged 2 years and older who have a rare, serious inherited condition called "spinal muscular atrophy" (SMA).

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

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

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, take other medicines, or are pregnant or breastfeeding.

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

3. What if my child is using other medicines?

Some medicines may interfere with Itvisma 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 Itvisma?

Itvisma is given to your child by a single intrathecal (IT) injection. IT is an injection directly into the space surrounding the spinal cord. 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 Itvisma, 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 Itvisma? in the full CMI.

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

Things you should do	• Keep all your doctor's appointments after your child receives Itvisma. • Keep all the appointments for your child's blood tests so that your doctor can monitor your child's health after Itvisma treatment.
After your child is given Itvisma	• The active medicine in Itvisma 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. • Continue to follow the handling instructions for at least 1 month after your child's treatment with Itvisma.

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

6. Are there any side effects?

There can be some serious side effects after Itvisma 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 and increased liver enzymes can occur with ITVISMA. If your child has a pre-existing liver condition before ITVISMA 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 ITVISMA. Liver function will be checked for at least 3 months after treatment.

Itvisma®

Active ingredient(s): *onasemnogene abeparvovec*

Consumer Medicine Information (CMI)

This leaflet provides important information about using Itvisma. **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 Itvisma.**

Where to find information in this leaflet:

1. Why is my child being given Itvisma?
2. What should I know before my child is given Itvisma?
3. What if my child is taking other medicines?
4. How will my child be given Itvisma?
5. What should I know after my child is given Itvisma?
6. Are there any side effects?
7. Product details

1. Why is my child being given Itvisma?

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

Itvisma is used to treat children aged 2 years of age and before reaching 19 years of age, 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. Itvisma can rescue viable motor neurons but does not rescue dead motor neurons.

How does Itvisma work?

Itvisma 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 Itvisma?

Warnings

Do not use Itvisma 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. Itvisma 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 Itvisma.
- 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 Itvisma 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** Itvisma treatment.

Prednisolone

Your child will also be given a medicine called prednisolone for a period of time as part of their treatment with Itvisma.

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 Itvisma. 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 them. See additional information under Section 6. Are there any side effects?

Use In Children

- Itvisma is suitable for use in children from 2 years of age with spinal muscular atrophy (SMA).
- The benefit of taking Itvisma 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 theoretical possibility that medicines like Itvisma can insert into the DNA of human body cells. As a result, Itvisma could cause tumours because of the nature of the medicine.

Pregnancy and breastfeeding

If you are pregnant or breastfeeding, think you might be pregnant, or are planning to have a baby, ask your doctor or healthcare provider for advice before being treated with Itvisma.

Females of child-bearing potential and male patients

If relevant your doctor will rule out pregnancy before Itvisma treatment. The effects of Itvisma on pregnancy and the unborn child are not known.

Women who could become pregnant should use effective birth control and should avoid egg donation for 6 months after receiving Itvisma treatment.

Men capable of fathering a child should use a condom and should avoid sperm donation for 3 months after receiving Itvisma treatment.

Ask your doctor or healthcare provider about effective birth control options.

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 Itvisma and affect how it works.

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

4. How will my child be given Itvisma?

How much will be given

- Itvisma will be given to your child ONCE only.
- The usual dose is 1.2 x e14 vg

When will Itvisma be given

- Your child will also be given treatment with prednisolone by mouth (or another corticosteroid), starting 24 hours before starting Itvisma. The dose of corticosteroid will 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 Itvisma be given

- Itvisma will be given to your child by a doctor or nurse experienced in treating SMA
- Itvisma is given by injection into the lower back. This injection, called a lumbar puncture, is done by inserting a needle into the space around the spinal cord. This will be done by a doctor or healthcare provider experienced in doing lumbar punctures. Your child may also be given a medicine to make them relax or sleep during the procedure.

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

Your child will be given prednisolone (or another corticosteroid) daily for approximately 2 months after the dose of Itvisma, 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 Itvisma.

The active medicine in Itvisma 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 Itvisma. Talk to your child's doctor or nurse if you have any questions.

If you have any further questions on the use of Itvisma 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.
- Itvisma may lower the blood platelet count (thrombocytopenia). This has been observed to generally occur within the first two weeks after Itvisma treatment. Your child will have regular blood tests to monitor changes in blood platelets for up to 3 months after infusion.
- Your child will have a blood test to check the amount of blood cells (red blood cells and cells involved in clotting) and creatinine level which shows how well the kidneys are working.

Call your doctor straight away if you notice:

- Yellowing of the skin or whites of the eyes, vomiting or decreased alertness.
- Signs of an infection (e.g. cold, flu or bronchiolitis).
- Bruising easily, seizures (fits) or decrease in urine output. This has been observed to generally occur within the first two weeks after Itvisma treatment (signs of abnormal blood clotting in small blood vessels (thrombotic microangiopathy), with decreased red blood cells and cells involved in clotting.
- Bruising or bleeding for longer than usual if your child has been hurt (signs of a low blood count/thrombocytopenia).
- Symptoms such as numbness, tingling, prickling or pain in the arms, hands, legs and/or feet. Sensations of pain, temperature and touch, indicating nerve problems could be a sign of peripheral sensory neuropathy.

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

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
• Nose and throat infection • Fever • Vomiting • Headache • Pain in arms/legs • Dizziness	**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. • Nerve problems affecting sensation of pain, temperature, touch or tingling in the arms, hands, legs and/or feet. • Yellowing of the skin or whites of the eyes, vomiting or decreased alertness	**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

Itvisma is only given in a hospital.

What Itvisma contains

Active ingredient (main ingredient)	Onasemnogene abeparvovec
Other ingredients (inactive ingredients)	Trometamol Magnesium chloride hexahydrate Sodium chloride Poloxamer Hydrochloric acid (for pH adjustment)

	Water for injections

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

What Itvisma looks like

Itvisma is supplied in a vial with a stopper, seal and coloured cap. The amount of liquid in each vial is 3 mL.

Itvisma is a clear to slightly opaque, colourless to faint white solution (AUST R 513645)

Who distributes Itvisma

Itvisma is supplied in Australia by:

Novartis Pharmaceuticals Australia Pty Limited

(ABN 18 004 244 160)

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Telephone 1 800 671 203

Website: www.novartis.com.au

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