ZAVESCA®

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.

1. Why am I using ZAVESCA?

ZAVESCA contains the active ingredient miglustat. ZAVESCA is used to treat mild to moderate Type 1 Gaucher disease in adults and it is also used to treat Niemann-Pick type C (NP-C) disease in children, adolescents and adults. For more information, see Section 1. Why am I using ZAVESCA? in the full CMI.

2. What should I know before I use ZAVESCA?

Do not use if you have ever had an allergic reaction to ZAVESCA or any of the ingredients listed at the end of the CMI. **Talk to your doctor if you have any other medical conditions, take any other medicines, or are pregnant or plan to become pregnant or are breastfeeding.** For more information, see Section 2. What should I know before I use ZAVESCA? in the full CMI.

3. What if I am taking other medicines?

Some medicines may interfere with ZAVESCA and affect how it works. A list of these medicines is in Section 3. What if I am taking other medicines? in the full CMI.

4. How do I use ZAVESCA?

- For Type 1 Gaucher disease, the usual dose for adults is one capsule (100 mg) three times a day (morning, afternoon and evening).
- For Niemann-Pick type C disease, the usual dose for adults and adolescents is two capsules (200 mg) three times a day (morning, afternoon and evening). Your doctor will work out the dose for children under the age of 12 years. If you have a problem with your kidneys you may receive a lower starting dose.
- ZAVESCA should be swallowed whole with water. It is recommended to take ZAVESCA without food.

More instructions can be found in Section 4. How do I use ZAVESCA? in the full CMI.

5. What should I know while using ZAVESCA?

Things you should do	• Remind any doctor, dentist or pharmacist you visit that you are using ZAVESCA. • **Tell your doctor if you become pregnant while you are taking ZAVESCA.** • Men and women should use effective birth control while taking ZAVESCA. • Men should keep using effective birth control for 3 months after treatment with ZAVESCA is stopped.
Things you should not do	• Do not get pregnant or breastfeed while you are taking ZAVESCA.
Driving or using machines	• ZAVESCA may make you feel dizzy. Do not drive or operate any tools or machines if you feel dizzy.
Looking after your medicine	• Store below 30°C. Keep your capsules in the blister pack out of reach of children until it is time to take them.

For more information, see Section 5. What should I know while using ZAVESCA? in the full CMI.

6. Are there any side effects?

Common, less serious side effects include abnormal blood test results which may cause symptoms like easy or excessive bruising or bleeding and fatigue, stomach complaints (e.g. diarrhoea, wind, abdominal pain, vomiting), weight loss or decreased appetite, lactose intolerance, insomnia, depression, headache, dizziness, unclear speech, muscle spasms, joint or bone pain, loss of strength, fatigue or feeling weak, chills, chest pain, rash, blurry vision, frequent, abnormal urination.

Serious side effects include tremor, tingling, numbness or pain in your hands and feet, memory or thinking problems.

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.

ZAVESCA®

Active ingredient: miglustat

Consumer Medicine Information (CMI)

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

Where to find information in this leaflet:

1. Why am I using ZAVESCA?
2. What should I know before I use ZAVESCA?
3. What if I am taking other medicines?
4. How do I use ZAVESCA?
5. What should I know while using ZAVESCA?
6. Are there any side effects?
7. Product details

1. Why am I using ZAVESCA?

ZAVESCA contains the active ingredient miglustat. ZAVESCA works by inhibiting the enzyme called 'glucosylceramide synthase' which is responsible for the first step in the synthesis of most glycosphingolipids.

ZAVESCA is used to treat mild to moderate Type 1 Gaucher disease in adults and it is also used to treat Niemann-Pick type C (NP-C) disease in children, adolescents and adults.

Type 1 Gaucher disease is an inherited disease that you get from both of your parents. People with Type 1 Gaucher disease are partially missing an enzyme that breaks down a chemical in the body called glucosylceramide. Too much glucosylceramide causes an increase in the size of the liver and spleen. It also causes bone disease and changes in the blood.

ZAVESCA reduces the formation of glucosylceramide to a level the partially missing enzyme can cope with.

You may be prescribed ZAVESCA if your doctor decides other treatments for this disease are not right for you.

Niemann-Pick disease is an inherited disease that you get from both parents. If you have NP-C, fats (glycosphingolipids) build up in the cells of your brain. This can result in disturbances in neurological functions such as eye movements, balance, swallowing, and memory, and in seizures.

2. What should I know before I use ZAVESCA?

Warnings

Do not use ZAVESCA if:

- you are allergic to miglustat, or any of the ingredients listed at the end of this leaflet.
- Always check the ingredients to make sure you can use this medicine.

Check with your doctor if you:

- have or have had any other medical conditions, especially the following:
- kidney disease
- liver disease
- take any medicines for any other condition

Do not take ZAVESCA if you suffer from severe kidney disease.

Your doctor may perform the following tests before treatment and during treatment with ZAVESCA:

- an examination to check the nerves in your arms and legs
- a test of your memory and thinking ability
- blood test to check your platelet count
- measurement of vitamin B12 levels (for GD-1 only)
- if you are a child or adolescent with NP-C your doctor will also check measurements of your growth

The reason these tests are done is that some patients have had tingling or numbness in their hands and feet, or memory and thinking problems, while taking ZAVESCA. The tests will help your doctor decide whether these effects are due to existing conditions, or due to side effects of ZAVESCA.

During treatment, you 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?

Pregnancy and breastfeeding

Check with your doctor if you are pregnant or intend to become pregnant.

Do not take ZAVESCA if you are pregnant or planning to become pregnant.

ZAVESCA may harm your baby. You must use effective birth control while you are taking ZAVESCA.

Talk to your doctor if you are breastfeeding or intend to breastfeed.

Do not take ZAVESCA if you are breastfeeding.

It is not known if this medicine passes into breast milk and if it can harm your baby.

ZAVESCA may harm sperm.

All men should use effective birth control while taking this medicine and for 3 months after they stop taking it.

3. What if I am taking other medicines?

Tell your doctor or pharmacist if you are 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 ZAVESCA and affect how it works.

Check with your doctor or pharmacist if you are not sure about what medicines, vitamins or supplements you are taking and if these affect ZAVESCA.

4. How do I use ZAVESCA?

How much to take

Type 1 Gaucher disease

- The usual dose for adults is one capsule (100 mg) three times a day (morning, afternoon and evening).

Niemann-Pick type C disease

- The usual dose for adults and adolescents is two capsules (200 mg) three times a day (morning, afternoon and evening).
- For children under the age of 12 years, the dose is determined by body surface area (BSA m2); your doctor will work this out for you.
- If you have a problem with your kidneys you may receive a lower starting dose.

Follow the instructions exactly as your doctor has instructed you to do and use ZAVESCA until your doctor tells you to stop.

When to take ZAVESCA

- Take ZAVESCA at the same time(s) each day.
- You must take ZAVESCA every day.
- Continue taking the medicine for as long as your doctor tells you to.
- Do not stop taking it unless your doctor tells you to even if you feel better.

How to take ZAVESCA

- ZAVESCA should be swallowed whole with water. It is recommended to take ZAVESCA without food.

If you forget to use ZAVESCA

ZAVESCA should be used regularly at the same time each day.

If it is almost time for your next dose, skip the dose you missed and take your next dose when you are meant to.

Otherwise, take it as soon as you remember and then go back to taking it as you would normally.

Do not take a double dose to make up for the dose you missed.

If you use too much ZAVESCA

If you think that you have used too much ZAVESCA, you may need urgent medical attention.

You should immediately:

- phone the Poisons Information Centre (**in Australia telephone 13 11 26 and in New Zealand telephone 0800 POISON or 0800 764 766)**, or
- contact your doctor, or
- go to the Emergency Department at your nearest hospital.

You should do this even if there are no signs of discomfort or poisoning.

5. What should I know while using ZAVESCA?

Things you should do

Tell your doctor if you become pregnant while you are taking ZAVESCA.

Men and women should use effective birth control while taking ZAVESCA.

Men should keep using effective birth control for 3 months after treatment with ZAVESCA is stopped.

Tell all the doctors, dentists and pharmacists who are treating you that you are taking ZAVESCA.

If you have diarrhoea your doctor may ask you to change your diet to reduce your lactose and carbohydrate intake such as sucrose (cane sugar), or not to take ZAVESCA together with food, or to temporarily reduce your dose. In some cases, the doctor may prescribe anti-diarrhoeal medications such as loperamide.

Cases of Crohn's disease (an inflammatory disease affecting the gut) have been reported in patients with Niemann-Pick type C disease treated with ZAVESCA.

If your diarrhoea does not respond to these measures, or if you have any other abdominal complaint, consult your doctor. In such a case, your doctor may decide to conduct further investigations.

Keep all of your doctor or clinic appointments.

Your doctor may do certain tests, including blood tests, from time to time to make sure the medicine is working and to prevent unwanted side effects.

You may get some weight loss when you start treatment with ZAVESCA. If you do lose some weight when you start treatment with ZAVESCA, don't worry. People usually stop losing weight as treatment goes on. Talk to your doctor if this worries you.

Things you should not do

- Do not get pregnant while you are taking ZAVESCA.
- Do not breastfeed while on ZAVESCA.
- Do not give ZAVESCA to anyone else, even if they have the same condition as you.
- Do not stop taking ZAVESCA or change your dose without checking with your doctor.

Driving or using machines

Be careful before you drive or use any machines or tools until you know how ZAVESCA affects you.

ZAVESCA may make you feel dizzy. Do not drive or operate any tools or machines if you feel dizzy.

Looking after your medicine

- Store below 30°C. Keep your capsules in the blister pack until it is time to take them.
- Follow the instructions in the carton on how to take care of your medicine properly.

Store it in a cool dry place away from moisture, heat or sunlight; for example, do not store it:

- in the bathroom or near a sink, or
- in the car or on window sills.

Keep it where young children cannot reach it.

Getting rid of any unwanted medicine

If you no longer need to use this medicine or it is out of date, take it to any pharmacy for safe disposal.

Do not use this medicine after the expiry date.

6. Are there any side effects?

All medicines can have side effects. If you do experience 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
Blood and lymphatic system-related • low platelet count (thrombocytopenia) causing symptoms like easy or excessive bruising and bleeding • low red blood cell count (anaemia) causing symptoms like fatigue, weakness, pale skin • swelling spleen or liver causing symptoms like abdominal pain, tiredness, yellowing of the skin and the whites of the eyes • abnormal blood test results **Gut-related** • diarrhoea (sometimes with blood) • stomach pain or discomfort, abdominal swelling or wind (flatulence) • feeling sick (nausea) • vomiting • constipation • heartburn/ indigestion • dry mouth • burping • inability to control bowel movement **Metabolism and nutrition-related** • weight loss • decreased or lack of appetite (anorexia) • lactose intolerance **Mental health-related** • difficulty in falling asleep (insomnia) • depression **Nervous system-related** • headache or migraine • dizziness • reduced sensation to touch (hypoesthesia) • loss of coordination of muscles (ataxia) • unclear speech **Muscle-related** • muscle spasms or weakness • joint or bone pain **General symptoms** • loss of strength (asthenia) • tiredness (fatigue) or feeling of weakness • chills • chest pain • feeling anxious • thirsty • increase in energy **Skin-related** • rash **Eye-related** • blurry vision **Urinary system-related** • frequent, abnormal urination	**Speak to your doctor if you have any of these less serious side effects and they worry you.**

Serious side effects

Serious side effects	What to do
• tremor usually in your hands • tingling, numbness or pain in your hands and feet • memory or thinking problems	**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 you feel unwell.

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

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.

Always make sure you speak to your doctor or pharmacist before you decide to stop taking any of your medicines.

7. Product details

This medicine is only available with a doctor's prescription.

What ZAVESCA contains

Active ingredient (main ingredient)	miglustat
Other ingredients (inactive ingredients)	sodium starch glycollate povidone magnesium stearate empty hard gelatin capsules size 4 white Op. 44.000/44.000 pure bovine (ARTG PI No 108031) OPACODE monogramming ink S-1-277002 black (ARTG PI No 107581), OPACODE monogramming ink S-1-27794 black (ARTG PI No 12104) or TekPrint SW-9008 Black Ink (ARTG PI No 2328)

Do not take this medicine if you are allergic to any of these ingredients.

What ZAVESCA looks like

ZAVESCA are white, hard capsules with "OGT 918" printed in black on the cap and "100" printed in black on the body.

ZAVESCA are supplied in blister packs of 90 capsules (AUST R 122957).

Who distributes ZAVESCA

JANSSEN-CILAG Pty Ltd

17 Khartoum Road

Macquarie Park NSW 2113 Australia

Telephone: 1800 226 334

NZ Office: Auckland New Zealand

Telephone: 0800 800 806

This leaflet was prepared in February 2026.

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