

Understanding your ZAVESCA® (miglustat) therapy

What is ZAVESCA?

ZAVESCA is a prescription medicine taken by mouth for adults with mild to moderate type 1 Gaucher disease. ZAVESCA is used only in people who cannot be treated with enzyme replacement therapy. It is not known if ZAVESCA is safe and effective in children under 18 years of age.



Who should not take ZAVESCA?

- **Do not take ZAVESCA if you are allergic to any of its ingredients. The active ingredient is miglustat.**
- **Do not take ZAVESCA if you are pregnant or may become pregnant while taking ZAVESCA.**

Please see Important Safety Information throughout this brochure and on back cover and accompanying full prescribing information, including Patient Information.



how it works

Your doctor has prescribed ZAVESCA® (miglustat) to help treat your type 1 Gaucher disease. This booklet will help you understand how ZAVESCA works and how it may help you. It also provides recommendations on different ways you can help yourself make the most of your medicine. Talk to your doctor if you have any questions about your disease or ZAVESCA.

What should I tell my doctor before taking ZAVESCA?

Before you take ZAVESCA tell your doctor if you:

- **Are pregnant or planning to become pregnant.**

ZAVESCA may harm your baby. You should use effective birth control while taking ZAVESCA.

ZAVESCA may also harm a man's sperm.

All men should use effective birth control during treatment with ZAVESCA and for 3 months after stopping ZAVESCA.

- **Are breastfeeding.** It is not known if ZAVESCA passes into your milk and if it can harm your baby. You should decide either to breast feed or take ZAVESCA, but not both.

- **Have kidney problems**

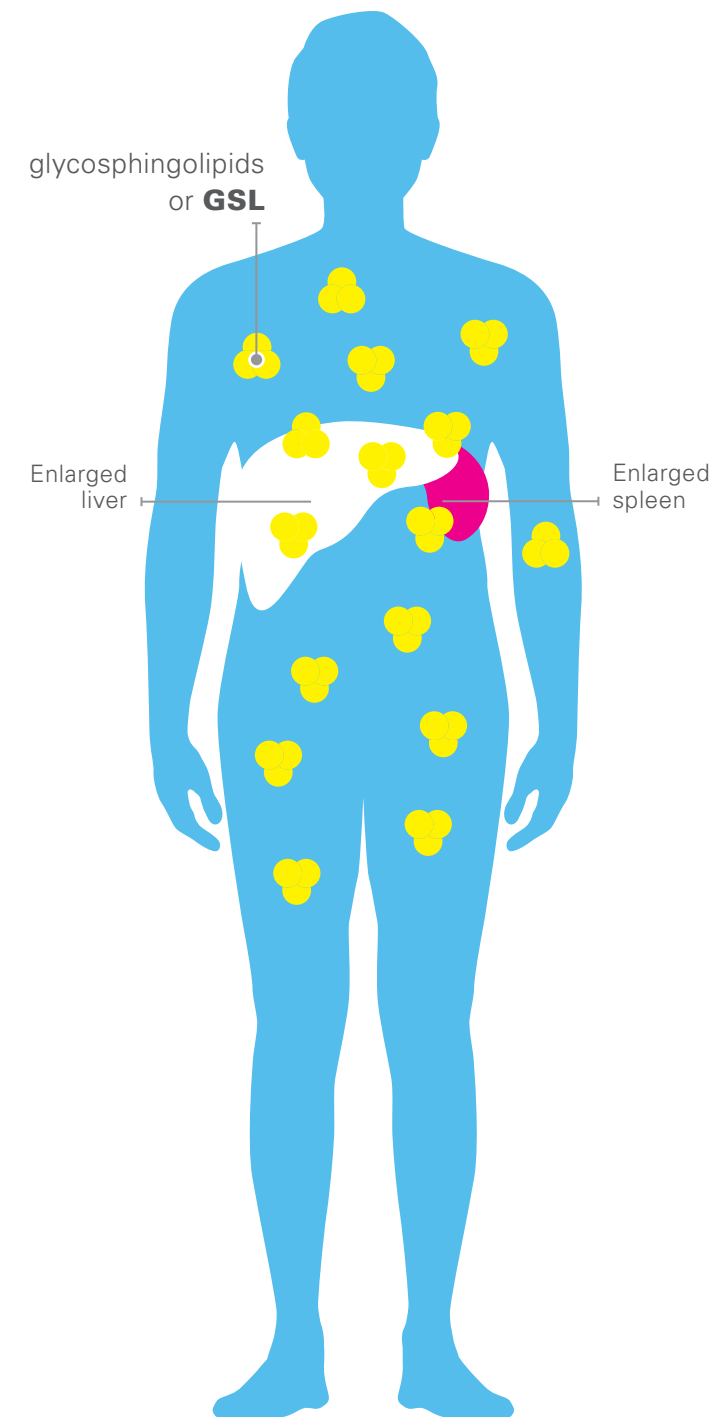
- **Have any other medical conditions**

Tell your doctor about all the medicines you take including prescription and non-prescription medicines, vitamins and other dietary supplements.

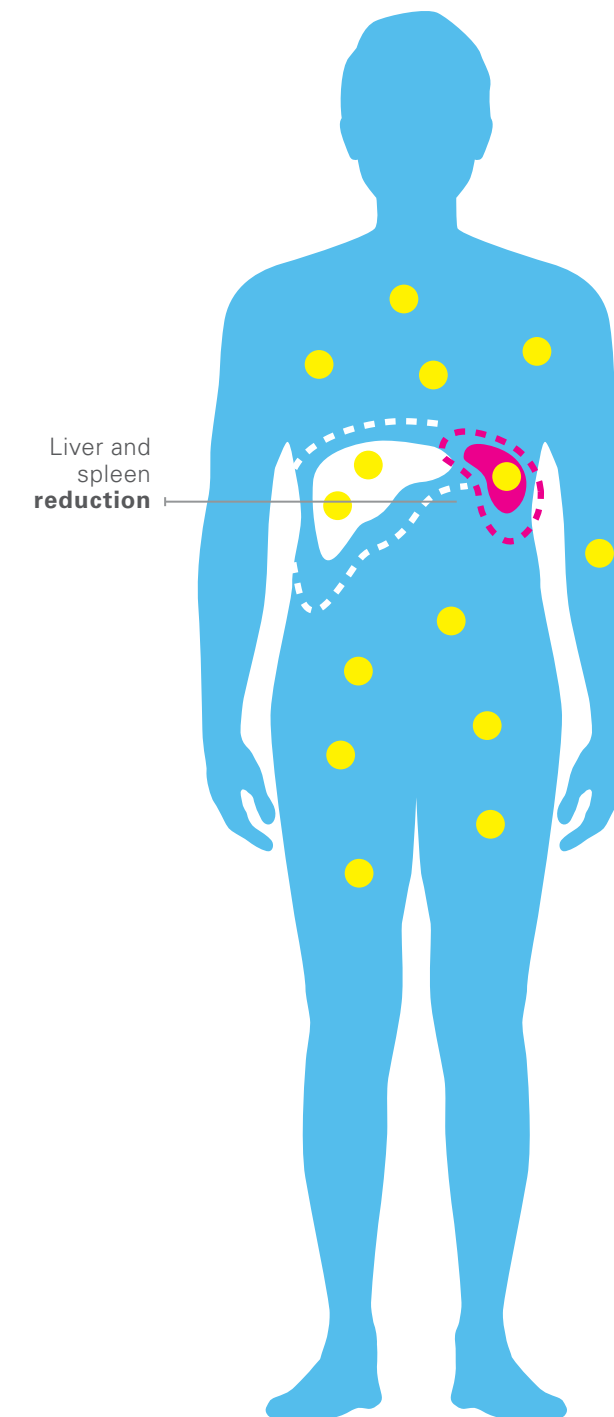
Some medicines may affect ZAVESCA. ZAVESCA may affect other medicines.

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before treatment



after ZAVESCA® (miglustat)



ZAVESCA® (miglustat) reduces the buildup of fatty substances by reducing the amount of glycosphingolipids (GSL) the body produces.*¹ This way, the enzymes you do have are able to break down the fatty substances.

*Based on laboratory studies.





Type 1 Gaucher disease

In people with type 1 Gaucher disease, the body has an excess of certain types of fatty substances called glycosphingolipids (gli-coh-sfing-goh-lip-ids) or GSL.

Normally, the body produces GSL and a certain enzyme breaks it down.

In people with type 1 Gaucher disease, there’s either not enough of the enzyme or it doesn’t work properly, so the fatty substances build up over time, causing serious problems in parts of the body like the liver and spleen. They may also have lowered levels of hemoglobin (a substance in red blood cells) and platelets (blood-clotting cells) that may cause anemia (low red blood cell count).

key benefits

ZAVESCA® (miglustat) reduces the harmful buildup of glycosphingolipids throughout the body to help treat some of the symptoms of mild to moderate type 1 Gaucher disease.

Clinical studies have shown that ZAVESCA provides important benefits for people with mild to moderate type 1 Gaucher disease, whether they have been treated before with enzyme replacement therapy (ERT) or they are being treated for the first time.¹ Individual results may vary.

ZAVESCA

- Reduced swelling of the liver and spleen¹
- Increased amounts of hemoglobin and platelets¹



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The most common side effects of ZAVESCA are:

- | | | |
|-------------------------|--------------------|------------------------------|
| • Stomach pain | • Weakness | • Heaviness in arms and legs |
| • Gas | • Vision problems | • Unsteady walking |
| • Nausea and vomiting | • Stomach bloating | • Memory loss |
| • Headache | • Back pain | • Decreased appetite |
| • Muscle and leg cramps | • Dry mouth | • Heartburn |
| • Dizziness | • Constipation | • Menstrual problems |

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side effects

ZAVESCA® (miglustat) may cause side effects. Many of these side effects, if they occur, may lessen or go away over time. Still, you should be aware of side effects that may occur while you are taking ZAVESCA.

What are the possible side effects of ZAVESCA® (miglustat)?

ZAVESCA may cause serious side effects including:

- **Problems affecting your nerves (neurologic problems):**
 - New or worse hand tremors (shaky movements).** Tremors may begin within the first month of starting treatment. Sometimes the tremors may go away between 1 to 3 months with continued treatment. Sometimes a lower dose or stopping ZAVESCA is needed to help the tremors go away. Call your doctor if you get hand tremors while taking ZAVESCA or the hand tremors you already have get worse.
 - Numbness and tingling in your hands, arms, legs, or feet (peripheral neuropathy).** Call your doctor right away if you get numbness or tingling in your arms or legs.
- Your doctor may test your nerves (neurological exam) before you start ZAVESCA and may repeat this procedure at a later time.
- **Diarrhea.** Your doctor may give you another medicine (anti-diarrheal) to treat diarrhea if it is a problem for you, and may recommend changes to your diet.
- **Weight loss.** You may also lose weight when you start treatment with ZAVESCA.
- **Low Platelet Count.** Your doctor may do blood tests to monitor your blood platelet count.

Diarrhea is the most common side effect for people taking ZAVESCA® (miglustat). Your doctor may give you a medicine to manage diarrhea if it is a problem for you, and may recommend dietary changes to reduce high-carbohydrate foods at the start of your treatment with ZAVESCA. Please see the “Simple Steps” and “Helping Yourself with Healthy Choices” brochures for guides to managing your diet. Your doctor or dietitian can also help you with developing a meal plan that is best for you.

Some of the other side effects that people have experienced with ZAVESCA are:

- Stomach pain
- Gas
- Nausea and vomiting
- Headache
- Muscle and leg cramps
- Dizziness
- Weakness
- Vision problems
- Stomach bloating
- Back pain
- Dry mouth
- Constipation
- Heaviness in arms and legs
- Unsteady walking
- Memory loss
- Decreased appetite
- Heartburn
- Menstrual problems

Call your doctor if you get any side effect that bothers you or that does not go away. Remember, sometimes the side effects will go away on their own. Your doctor will recommend a lower dose of ZAVESCA® (miglustat) if it is appropriate. You should not change your dose of ZAVESCA on your own. These are not all the side effects that may occur with ZAVESCA. For more information, ask your doctor or your pharmacist.

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making the most of

ZAVESCA® (miglustat) may provide benefits to people with type 1 Gaucher disease. Still, keep in mind that ZAVESCA may take some time to work in ways you can notice. Even though some people taking ZAVESCA may not feel better right away, they can experience benefits that their doctors can measure. Individual results may vary.



ZAVESCA therapy

Changing your diet can make a difference.

Some people who take ZAVESCA® (miglustat) may get gas, loose stools, or diarrhea. The reason is that ZAVESCA may slow your body's digestion of some carbohydrates, which can cause these issues. If this occurs with you, you can consider reducing your intake of carbohydrates and other high-starch foods and drinks that may be contributing to the gas, loose stools, and diarrhea. Carbohydrates are found in grains, bread, rice, and flour, as well as in the form of simple sugars in soda, cookies, and desserts.

After being on ZAVESCA for about a month, your body may adjust to therapy and you may be able to slowly bring certain foods back into your diet. Work with your doctor or dietitian to decide what is best for you.

Taking ZAVESCA® (miglustat)

It's important to take ZAVESCA as prescribed by your doctor, even if you are feeling better. The recommended dose of ZAVESCA is usually 1 pill (100 mg) 3 times a day, but your doctor will tell you which dose is right for you.

Giving it time

Remember, different people react to ZAVESCA in different ways.

Have patience and stick with it—ZAVESCA may help keep your disease under control.

See the “Helping Yourself with Healthy Choices” brochure for diet recommendations, and be sure to keep your doctor informed about how you feel.

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patient support

ZAVESCA® (miglustat) is distributed by CuraScript, Inc., a specialty pharmacy that ensures that patients have continued access to their medication.

CuraScript Patient Support

CuraScript's ZAVESCA® (miglustat) team is made up of registered nurses and pharmacists who are available to answer your questions about ZAVESCA, including:

- What to expect from ZAVESCA treatment
- Making the most of your treatment
- Shipping information
- Managing diet while taking ZAVESCA

If you have questions about your ZAVESCA treatment, call CuraScript's ZAVESCA Team Monday through Friday, 8 AM to 6 PM Eastern time (5 AM to 3 PM Pacific time). Even after hours, there is always a CuraScript representative on call to help you.

CuraScript ZAVESCA Team
1-888-281-5582

Be sure to ask the CuraScript ZAVESCA® (miglustat) Team whether you are eligible for ZAVESCA Financial Support

- A financial assistance program available to eligible people taking ZAVESCA
- Co-pay and out-of-pocket expenses may be covered
- The level of assistance may vary depending on whether or not you have insurance

Patients ineligible for financial support include, but are not limited to, those enrolled in Medicare, Medicaid, VA/DOD (Tricare), the Indian Health Service, or any other federally or state-funded healthcare program, or where prohibited by law.

The National Gaucher Foundation

National Gaucher Foundation
www.gaucherdisease.org
1-800-504-3189

**If you'd like to contact
Actelion Pharmaceuticals US,
Inc., call 1-866-ACTELION
(1-866-228-3546)**



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frequently asked questions

How is ZAVESCA® (miglustat) different from enzyme replacement therapy?

Currently, there are 2 ways to treat type 1 Gaucher disease. One way is to decrease the body’s over-accumulation of harmful fatty substances by limiting production of GSLs. The other way is enzyme replacement therapy (ERT), where man-made enzyme is added to the body to help break down the fatty substances. ZAVESCA is also different because, as a small molecule, it goes throughout the body.

Can ZAVESCA be used to treat type 1 Gaucher disease that has been previously treated with ERT?

Yes. Studies with ZAVESCA included both people who had previously taken Cerezyme (imiglucerase)—also called enzyme replacement therapy (ERT)—but had stopped taking ERT for at least 6 months, and people who had never been treated with ERT.¹

Within 6 months, people treated with ZAVESCA had improvements in 4 key areas:

- Reduction in the size of livers.¹
- Reduction in size of spleens.¹
- Platelet counts increased.¹
- Hemoglobin concentration increased.¹

Your doctor will generally monitor these 4 key areas in addition to other tests to see how your treatment is doing.

How do I take ZAVESCA® (miglustat)? When should I take ZAVESCA?

It’s important to take ZAVESCA as prescribed by your doctor. The recommended dose of ZAVESCA is usually 1 pill (100 mg) taken 3 times each day. In some cases, your doctor may have you take more or less, depending on what he or she thinks is right for you.

You should take ZAVESCA at the same time each day. For example, if your prescription is for ZAVESCA 3 times a day, then take it when you first wake up, in the early afternoon, and before going to bed (or as close to 8-hour intervals as possible).

You can take ZAVESCA with or without food. Some people experience fewer side effects when taking ZAVESCA without food, while others see the opposite effect. You can discuss with your doctor what works best for you.

What blood tests need to be monitored when I take ZAVESCA?

No blood tests are required to take ZAVESCA. However, your doctor may want to monitor your type 1 Gaucher disease activity by taking blood samples regularly to determine your levels of hemoglobin, platelet counts, and important disease markers (such as chitotriosidase, angiotensin-converting enzyme, and tartrate-resistant acid phosphatase). Your doctor will let you know how often to have these blood tests.



Who should not take ZAVESCA® (miglustat)? Who should be cautious about taking ZAVESCA?

You should NOT take ZAVESCA if you are pregnant or nursing because ZAVESCA can harm a fetus. Do not use ZAVESCA if you plan to become pregnant, or if your partner can become pregnant. You must use reliable birth control while you are taking ZAVESCA.

ZAVESCA may cause changes in sperm development and function. If you are a man, you should use reliable birth control while you are taking ZAVESCA and you should not father a child for at least 3 months after you stop taking ZAVESCA.

It’s important to talk to your doctor if any of the following apply to you:

- If you are breastfeeding. It is not known if ZAVESCA passes into your milk, or if it can harm your baby. You should decide either to breast feed or take ZAVESCA, but not both.
- If you have kidney problems. There are special recommendations for taking ZAVESCA, so your doctor may prescribe a different dose if you have kidney problems.
- If you have existing neuropathy or a disease that causes tremor, like Parkinson’s disease.
- If you are lactose intolerant or have other forms of chronic diarrhea.

ZAVESCA® (miglustat) has not been studied in patients with type 1 Gaucher disease under 18 years of age.

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INDICATION AND IMPORTANT SAFETY INFORMATION

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- **Are breastfeeding.** It is not known if ZAVESCA passes into your milk and if it can harm your baby. You should decide either to breast feed or take ZAVESCA, but not both.

- **Have kidney problems**
- **Have any other medical conditions**

Tell your doctor about all the medicines you take including prescription and non-prescription medicines, vitamins and other dietary supplements. Some medicines may affect ZAVESCA. ZAVESCA may affect other medicines.

How should I take ZAVESCA?

- Take ZAVESCA exactly as your doctor has prescribed. Check with your doctor or your pharmacist if you are not sure.

What are the possible side effects of ZAVESCA?

ZAVESCA may cause serious side effects including:

- **Problems affecting your nerves (neurologic problems):**

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Numbness and tingling in your hands, arms, legs, or feet (peripheral neuropathy).

Call your doctor right away if you get numbness or tingling in your arms or legs.

Your doctor may test your nerves (neurological exam) before you start ZAVESCA and may repeat this procedure at a later time.

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- Muscle and leg cramps
- Dizziness
- Weakness
- Vision problems
- Stomach bloating
- Back pain
- Dry mouth
- Constipation
- Heaviness in arms and legs
- Unsteady walking
- Memory loss
- Decreased appetite
- Heartburn
- Menstrual problems

Call your doctor if you have any side effect that bothers you or that does not go away. These are not all the side effects with ZAVESCA. For more information, ask your doctor or your pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to the FDA at **1-800-FDA-1088**.

Please see accompanying full prescribing information, including Patient Information.



www.actelion.com

References: **1.** ZAVESCA (miglustat) full prescribing information. Actelion Pharmaceuticals US, Inc. November 2010. **2.** Alfonso P, Pampin S, Estrada J, et al. Miglustat (NB-DNJ) works as a chaperone for mutated acid β -glucosidase in cells transfected with several Gaucher disease mutations. *Blood Cell Mol Dis.* 2005;35:268-276.

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