



A Guide to Treatment With ZAVESCA[®]

ZAVESCA[®], when used alone, can make a difference in treating type 1 Gaucher disease in people who cannot be treated with enzyme replacement therapy.¹

INDICATION AND IMPORTANT SAFETY INFORMATION

What is ZAVESCA® (miglustat)?

ZAVESCA® is a prescription medicine used alone to treat 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.

What should I tell my doctor before taking ZAVESCA®?

Before you take ZAVESCA® tell your doctor if you:

- have kidney problems
- have any other medical conditions
- are pregnant or plan to become pregnant. It is not known if ZAVESCA® will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if ZAVESCA® passes into your breast milk. You and your doctor should decide if you will breastfeed or take ZAVESCA®. You should not do both.

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. ZAVESCA® may affect how other medicines work.

Know the medicines you take. Keep a list of them to show your doctor and pharmacist when you get a new medicine.

How should I take ZAVESCA®?

- Take ZAVESCA® exactly as your doctor has prescribed.
- Take ZAVESCA® at the same time each day.
- If you miss a dose of ZAVESCA®, skip that dose. Take the next ZAVESCA® capsule at the usual time.

What are the possible side effects of ZAVESCA®?

ZAVESCA® may cause serious side effects including:

- **Numbness, tingling, pain, or burning in your hands or feet (peripheral neuropathy).** Call your doctor right away if you get numbness, tingling, pain, or burning in your hands or feet.
- Your doctor may test your nerves (neurological exam) before you start ZAVESCA® and during treatment with ZAVESCA®.
- **New or worsening hand tremors (shaky movements).** Tremors are common with ZAVESCA® and may begin within the first month of starting

treatment. Sometimes the tremors may go away between 1 to 3 months with continued treatment. Your doctor may lower your dose or stop ZAVESCA® if you develop new or worsening hand tremors. Call your doctor right away if you get new hand tremors while taking ZAVESCA® or if the hand tremors you already have get worse.

- **Diarrhea** is common with ZAVESCA® and sometimes can be serious. Your doctor may prescribe another medicine (anti-diarrheal) to treat diarrhea if it is a problem for you and may recommend changes to your diet, such as avoiding foods high in carbohydrates. Talk with your doctor about your diet if you have diarrhea.
- **Weight loss** is common with ZAVESCA® and sometimes can be serious. You may lose weight when you start treatment with ZAVESCA®.
- **Low platelet count** is common with ZAVESCA® and can be serious. Your doctor may do blood tests to monitor your blood platelet count.

The most common side effects of ZAVESCA® are:

- | | |
|--------------------------------|---|
| • Weight loss | • Dry mouth |
| • Stomach pain | • Heaviness in arms and legs |
| • Gas | • Memory loss |
| • Nausea and vomiting | • Unsteady walking |
| • Headache, including migraine | • Loss of appetite |
| • Leg cramps | • Indigestion |
| • Dizziness | • Numbness, tingling, pain, or burning of your skin |
| • Weakness | • Stomach bloating |
| • Vision problems | • Stomach pain not related to food |
| • Muscle cramps | • Menstrual changes |
| • Back pain | |
| • Constipation | |

Tell your doctor if you have any side effect that bothers you or that does not go away.

These are not all the possible side effects of ZAVESCA®. For more information, ask your doctor or pharmacist.

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

Please see full Prescribing Information, including Patient Information, at www.zavesca.com.

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What is ZAVESCA®?	Type 1 Gaucher Disease	How ZAVESCA® Works	How ZAVESCA® is Taken	Managing Possible Side Effects
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Get Ready to Start Treatment

This booklet is designed to introduce some important things you’ll want to know about treatment with ZAVESCA® (miglustat).

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What is ZAVESCA®?

Type 1
Gaucher Disease

How ZAVESCA®
Works

How ZAVESCA®
is Taken

Managing Possible
Side Effects

An oral therapy that can be used alone¹

ZAVESCA® (miglustat) is a pill indicated for the treatment of adults with mild to moderate type 1 Gaucher disease who cannot be treated with enzyme replacement therapy (ERT).¹



ZAVESCA® can be used to treat type 1 Gaucher disease that has been previously treated with ERT¹



The label does not require a blood test to determine your eligibility to take ZAVESCA®





What is ZAVESCA®?

Type 1
Gaucher Disease

How ZAVESCA®
Works

How ZAVESCA®
is Taken

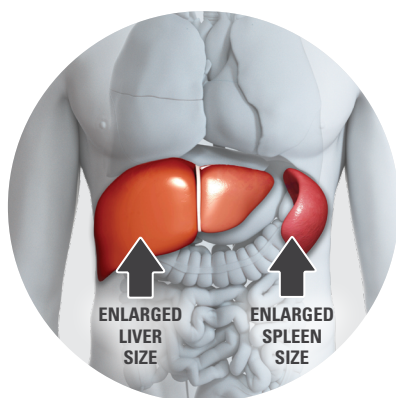
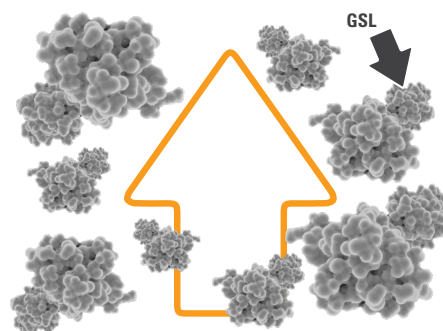
Managing Possible
Side Effects

Type 1 Gaucher Disease

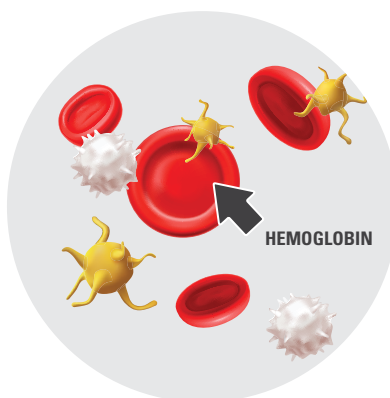
In people with type 1 Gaucher disease, the body has an excess of certain types of fatty substances called **glycosphingolipids (gli-coh-sfing-goh-lip-ids) or GSLs.**^{2,3}

There's either not enough of the enzyme that breaks down GSLs or it is not working properly, leading to these fatty substances building up over time.¹

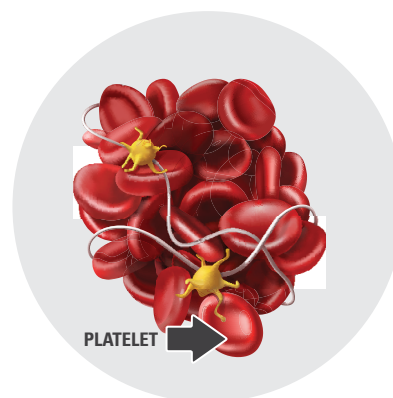
This can cause serious problems, such as:



Liver and spleen
enlargement³



Anemia (can be indicated by
lowered levels of hemoglobin)^{3,4}



Lowered levels of
platelets³



What is ZAVESCA®?

Type 1
Gaucher Disease

How ZAVESCA®
Works

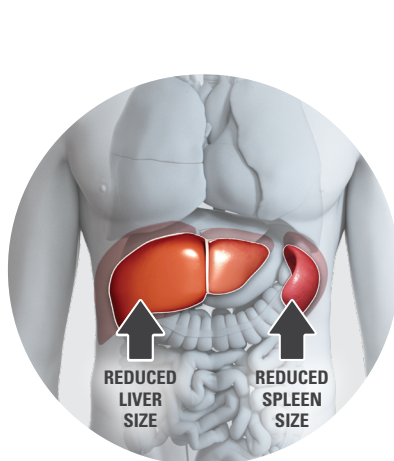
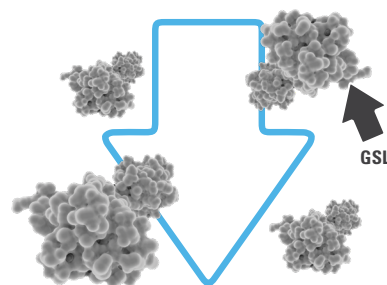
How ZAVESCA®
is Taken

Managing Possible
Side Effects

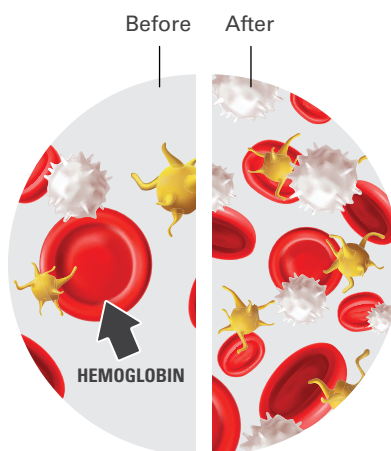
ZAVESCA® (miglustat) provides measurable benefits

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

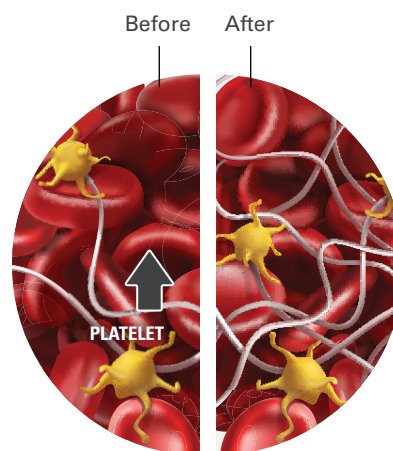
Clinical studies have shown that ZAVESCA® provides important benefits for adults with mild to moderate type 1 Gaucher disease^{1*}:



Reduced swelling of
liver and spleen



Increased levels of
hemoglobin



Increased levels of
platelets

*Individual results may vary.



What is ZAVESCA®?

Type 1
Gaucher Disease

How ZAVESCA®
Works

How ZAVESCA®
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Managing Possible
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How to Take ZAVESCA® (miglustat)



Take ZAVESCA® as prescribed by your doctor¹

Although some people may not feel better right away, they can still experience benefits that their doctors can measure.



Take ZAVESCA® at the same time each day¹

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.



If you miss a dose of ZAVESCA®, skip that dose¹

Take the next ZAVESCA® capsule at the usual time.



Recommended ZAVESCA® Dosage

One 100-mg capsule 3 times daily at regular intervals.¹



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Type 1
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How ZAVESCA®
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Managing Possible
Side Effects

Diarrhea is a common side effect for people taking ZAVESCA® (miglustat).¹

Some people who take ZAVESCA® may get gas, loose stools, or diarrhea.^{1,5} This is because ZAVESCA® may slow your body's digestion of some carbohydrates.⁵

In clinical trials, diarrhea decreased over time with continued ZAVESCA® treatment.¹

Talk to your healthcare provider about additional steps that you can take to prevent or help reduce your symptoms, including some of the steps below:



Watch what you eat

Your doctor may instruct you to modify your diet at the start of treatment.

- Avoid refined carbohydrates, reduce your intake of starchy or sugary foods, and regulate dairy consumption⁵
- **After being on ZAVESCA® for a month, you may be able to slowly bring certain foods back into your diet as your body adjusts to therapy with ZAVESCA®. Discuss timing with your doctor.**



Take ZAVESCA® in between meals¹



Ask your doctor about an anti-diarrheal medication

In clinical studies, patients received loperamide for their diarrhea symptoms^{1,5}

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Talk with your healthcare provider



Talk to your healthcare provider to find out if ZAVESCA® (miglustat) may be right for you.

To learn more and download additional resources, visit www.zavesca.com

References: 1. ZAVESCA® (miglustat) full prescribing information. Actelion Pharmaceuticals US, Inc. 2. Cox T, Lachmann R, Hollak C, et al. Novel oral treatment of Gaucher's disease with N-butyldeoxynojirimycin (OGT 918) to decrease substrate biosynthesis. *Lancet*. 2000;355:1481-1485. 3. Zeller JL, Burke AE, Glass RM. JAMA patient page. Gaucher disease. *JAMA*. 2007;298:1358. 4. World Health Organization. *Anaemia*. Accessed November 6, 2024. https://www.who.int/health-topics/anaemia#tab=tab_1 5. Belmatoug N, Burlina A, Giraldo P, et al. Gastrointestinal disturbances and their management in miglustat-treated patients. *J Inherit Metab Dis*. 2011;34:991-1001.