

What is ZAVESCA® (miglustat)?

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.

Simple steps
for making the most of
ZAVESCA



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.



understanding your

ZAVESCA® (miglustat) can make an important difference for those who have type 1 Gaucher disease. Your doctor has prescribed ZAVESCA for you or a loved one. This brochure is designed to help individuals and families understand what to expect while using 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.

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.

therapy

About ZAVESCA® (miglustat) therapy

ZAVESCA has been proven to deliver important benefits. Still, keep in mind that it may take some time to work in ways you can notice. Individual results may vary. Even though some people taking ZAVESCA may not feel better right away, they can experience benefits that their doctors can measure.¹

It's important for you or your loved one to take ZAVESCA as prescribed, usually 3 times each day.¹ And even when you feel better or experience benefits, you still need to keep taking ZAVESCA as prescribed to maintain the benefits.

So have patience and stick with it—ZAVESCA may help keep your disease under control.¹

Read about the benefits of ZAVESCA and Important Safety Information in the "Understanding Your ZAVESCA Therapy" brochure (included in your patient information folder) and at www.ZAVESCA.com.

What to do about possible side effects

ZAVESCA® (miglustat) has side effects. While they may go away on their own over time, people who take ZAVESCA should call their doctor if they become concerned about these, or other potential, side effects.¹

Diarrhea and **gas** are two of the most common side effects for people taking ZAVESCA.¹ Recommendations for managing diet to help minimize these effects can be found on the following pages.

Other serious side effects related to ZAVESCA include:

- **Weight loss** Some people lose weight when starting treatment with ZAVESCA.¹
- **Hand tremors** (Shaky movements like you've had too much coffee.) If hand tremors start or existing tremors get worse, you should call your doctor. He or she may lower the dose or stop ZAVESCA to help manage the tremors.¹
- **Numbness and tingling in hands, arms, legs, or feet (peripheral neuropathy)** Call your doctor right away if you experience any of these side effects.¹
- **Low Platelet Count.** Your doctor may do blood tests to monitor your blood platelet count.

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managing **diet** can make a difference

In people with type 1 Gaucher disease, the body has an excess of fatty substances called glycosphingolipids (gli-coh-sfing-goh-lip-ids).²

These fatty substances are harmful when they build up in cells.² ZAVESCA® (miglustat) works by limiting the body's production of these fatty substances, which helps control your disease.¹

What are the possible side effects of ZAVESCA?

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.

Just as it limits overproduction of these fatty substances, ZAVESCA® (miglustat) may also limit how the body digests carbohydrates.^{1,3} Carbohydrates are found in grains, bread, rice, and flour. Simple sugars, found in things like soda, cookies, or desserts, are also carbohydrates. ZAVESCA may affect how long carbohydrates stay in the intestines, so carbohydrates may be digested more slowly than normal and can cause diarrhea and gas.^{1,4}

While many people who take ZAVESCA may get gas, loose stools, or diarrhea, the incidence has been noted to decrease over time, and was noted to result in an increase in the use of anti-diarrheal medications, most commonly loperamide.¹

Taking the simple step of reducing carbohydrates in the diet may help manage these side effects. The recommendations inside can help you or your loved one make the most of therapy with ZAVESCA.

You may know people who have allergies to wheat or trouble digesting carbohydrates like grains. They help control their gas and bloating by watching their diet and avoiding certain foods. If you experience diarrhea or gas with ZAVESCA® (miglustat), a change in diet can often be very helpful. Your doctor may also recommend over-the-counter medications to help control gas and diarrhea.¹

Talk to your doctor or a dietitian to see what may work best. Make certain to follow the doctor's directions about how to take ZAVESCA.

Please see Important Safety Information throughout this brochure and on back cover.



Here are some dietary recommendations:

avoid



- Refined grains (white bread, white pasta, most breakfast cereals)
- Sugary foods (soft drinks, pastries, candy bars, sweets, desserts)
- Dairy products (ice cream, milk, butter, yogurt, sour cream, soft cheeses)
- Foods containing sorbitol and mannitol (sugary candies, hard candies)

reduce



- Starchy foods (potatoes, rice, whole grains)
- Fiber intake (no more than 4 grams per serving)
- Alcohol and drinks with caffeine

enjoy



- Most fruits
- Most vegetables
- Lactose-free products (soy milk)
- High-protein foods such as eggs, nuts, and seeds
- Lean meats
- Margarine or foods cooked with soybean or safflower oil

consider

Eating small healthy snacks between meals, especially if weight loss is a concern.

Taking ZAVESCA® (miglustat) at the same time each day—for instance, 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).

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

It’s important to remember that different people react to ZAVESCA® (miglustat) in different ways. 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. Work with your doctor to talk about when the time might be right for you.

For more recommendations on dietary management, including examples of specific foods to enjoy, reduce, and avoid, please see the accompanying brochure entitled “Helping Yourself to Healthy Choices.”

Talk to your doctor about taking an anti-diarrheal medication if diarrhea is a problem for you.¹

Talk to your doctor or a dietitian to see what may work best. Make certain to follow the doctor’s directions about how to take ZAVESCA.

Adapted from USDA Dietary Guidelines.
<http://ndb.nal.usda.gov/ndb/foods/list>

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understanding

For more information about ZAVESCA® (miglustat), ZAVESCA Financial Support, or CuraScript, contact CuraScript's dedicated ZAVESCA team.

The most common side effects of ZAVESCA are:

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

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**.

patient support

CuraScript

- ZAVESCA® (miglustat) is not available at your local pharmacy. CuraScript is a specialty pharmacy that sends ZAVESCA directly to you on the direction of your doctor and can provide information such as:
 - What to expect from ZAVESCA treatment
 - How to make the most of your treatment
- With CuraScript's services, no trips to the pharmacy are necessary to pick up your ZAVESCA prescription or order refills

CuraScript ZAVESCA Support
1-888-281-5582

ZAVESCA Financial Support

- Financial assistance may be 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.

Zavesca Cares™ is here to offer one-on-one individual assistance

- *Zavesca Cares* offers supplemental counseling and educational support services related to ZAVESCA® (miglustat) therapy for patients with type 1 Gaucher disease who are ages 18 and older
 - Brought to you by Actelion Pharmaceuticals US, Inc., the company that makes ZAVESCA
- Educators include a small team of trained nurses and dietitians to help answer your questions about ZAVESCA therapy, type 1 Gaucher disease, and dietary concerns
- To sign up, fill out the *Zavesca Cares* authorization in your welcome folder (also available online at www.ZAVESCA.com) and return it to Actelion, or call 1-855-426-9277

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)

References: 1. ZAVESCA® (miglustat) full prescribing information. Actelion Pharmaceuticals US, Inc. November 2010. 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. Andersson U, Butters TD, Dwek RA, Platt FM. *N*-butyldeoxygalactonojirimycin: a more selective inhibitor of glycosphingolipid biosynthesis than *N*-butyldeoxynojirimycin, in vitro and in vivo. *Biochem Pharmacol*. 2000;59:821-829. 4. Déchelotte P. Gastrointestinal and nutritional adverse effects of ZAVESCA (miglustat). Expert report, Actelion Pharmaceuticals Ltd. April 18, 2003.



INDICATION AND IMPORTANT SAFETY INFORMATION

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www.actelion.com

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- **Low Platelet Count.** Your doctor may do blood tests to monitor your blood platelet count.

The most common side effects of 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

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