

Miglustat

This information from UpToDate® Lexidrug™ explains what you need to know about this medication, including what it's used for, how to take it, its side effects, and when to call your healthcare provider.

Brand Names: US

Opfolda; Yargesa; Zavesca

Brand Names: Canada

Opfolda; SANDOZ Miglustat; Zavesca

What is this drug used for?

- It is used to treat Gaucher disease.
- If your child has been given this drug for some other reason, talk with the doctor about the benefits and risks. Talk with the doctor if you have questions or concerns about giving this drug to your child.

What do I need to tell the doctor BEFORE my child takes this drug?

- If your child is allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell the doctor about the allergy and what signs your child had.
- If your child has kidney disease.

If your child is breast-feeding a baby:

- Be sure your child does not breast-feed a baby while taking this drug.

This is not a list of all drugs or health problems that interact with this drug.

Tell the doctor and pharmacist about all of your child's drugs (prescription or OTC, natural products, vitamins) and health problems. You must check to make sure that it is safe to give this drug with all of your child's other drugs and health problems. Do not start, stop, or change the dose of any drug your child takes without checking with the doctor.

What are some things I need to know or do while my child takes this drug?

- Tell all of your child's health care providers that your child is taking this drug. This includes your child's doctors, nurses, pharmacists, and dentists.
- Your child may bleed more easily. Make sure your child is careful and avoids injury. Be sure your child has a soft toothbrush.
- Have your child's blood work and other lab tests checked as you have been told by the doctor.
- This drug may cause diarrhea. If your child gets diarrhea, talk with the doctor about ways to lower this effect. Do not stop giving this drug to your child without talking with the doctor.
- Have your child follow the diet plan your child's doctor told you about.
- This drug may affect growth in children and teens in some cases. They may need regular growth checks. Talk with the doctor.

If your child is pregnant:

- This drug may cause harm to the unborn baby if your child takes it during pregnancy. If your child is pregnant or gets pregnant while taking this drug, call the doctor right away.

What are some side effects that I need to call my child's doctor about right away?

WARNING/CAUTION: Even though it may be rare, some people may have very bad and sometimes deadly side effects when taking a drug. Tell your child's doctor or get medical help right away if your child has any of the following signs or symptoms that may be related to a very bad side effect:

- Signs of an allergic reaction, like rash; hives; itching; red, swollen, blistered, or peeling skin with or without fever; wheezing; tightness in the chest or throat; trouble breathing, swallowing, or talking; unusual hoarseness; or swelling of the mouth, face, lips, tongue, or throat.
- A burning, numbness, or tingling feeling that is not normal.
- Weakness.
- Change in eyesight.
- Any unexplained bruising or bleeding.
- Memory problems or loss.
- Trouble walking.

What are some other side effects of this drug?

All drugs may cause side effects. However, many people have no side effects or only have minor side effects. Call your child's doctor or get medical help if any of these side effects or any other side effects bother your child or do not go away:

- Dizziness or headache.
- Constipation, diarrhea, stomach pain, upset stomach, throwing up, or decreased appetite.
- Heartburn.
- Weight loss.
- Gas.
- Bloating.

- Dry mouth.
- Back pain.
- Leg cramps.
- Muscle cramps.
- Feeling of heaviness in your arms or legs.
- Period (menstrual) changes.
- Shakiness or tremors are common with drug. Sometimes, this may go away in 1 to 3 months. Call the doctor right away if your child has new or worsening hand shakiness or tremors.

These are not all of the side effects that may occur. If you have questions about side effects, call your child's doctor. Call your child's doctor for medical advice about side effects.

You may report side effects to your national health agency.

How is this drug best given?

Give this drug as ordered by your child's doctor. Read all information given to you. Follow all instructions closely.

- It is best to give this drug in between meals or as you have been told by your child's doctor.
- Give this drug at the same time of day.
- Keep giving this drug to your child as you have been told by your child's doctor or other health care provider, even if your child feels well.

What do I do if my child misses a dose?

- Skip the missed dose and go back to your child's normal time.
- Do not give 2 doses at the same time or extra doses.

How do I store and/or throw out this drug?

- Store at room temperature in a dry place. Do not store in a bathroom.
- Keep all drugs in a safe place. Keep all drugs out of the reach of children and pets.
- Throw away unused or expired drugs. Do not flush down a toilet or pour down a drain unless you are told to do so. Check with your pharmacist if you have questions about the best way to throw out drugs. There may be drug take-back programs in your area.

General drug facts

- If your child's symptoms or health problems do not get better or if they become worse, call your child's doctor.
- Do not share your child's drug with others and do not give anyone else's drug to your child.
- Some drugs may have another patient information leaflet. If you have any questions about this drug, please talk with your doctor, nurse, pharmacist, or other health care provider.
- If you think there has been an overdose, call your poison control center or get medical care right away. Be ready to tell or show what was taken, how much, and when it happened.

Consumer Information Use and Disclaimer

This generalized information is a limited summary of diagnosis, treatment, and/or medication information. It is not meant to be comprehensive and should be used as a tool to help the user understand and/or assess potential diagnostic and treatment options. It does NOT include all information about conditions, treatments, medications, side effects, or risks that may apply to a specific patient. It is not intended to be medical advice or a substitute for the medical advice, diagnosis, or treatment of a health care provider based on the health care provider's examination and assessment of a patient's specific and unique circumstances. Patients must speak with a health care provider for complete information about their health, medical questions, and treatment options,

including any risks or benefits regarding use of medications. This information does not endorse any treatments or medications as safe, effective, or approved for treating a specific patient. UpToDate, Inc. and its affiliates disclaim any warranty or liability relating to this information or the use thereof. The use of this information is governed by the Terms of Use, available at <https://www.wolterskluwer.com/en/know/clinical-effectiveness-terms>.

Last Reviewed Date

2025-07-29

Copyright

© 2026 UpToDate, Inc. and its affiliates and/or licensors. All rights reserved.

If you have questions or concerns, contact your healthcare provider. A member of your care team will answer Monday through Friday from 9 a.m. to 5 p.m. Outside those hours, you can leave a message or talk with another MSK provider. There is always a doctor or nurse on call. If you're not sure how to reach your healthcare provider, call 212-639-2000.

For more resources, visit www.mskcc.org/pe to search our virtual library.

Miglustat - Last updated on December 12, 2022

All rights owned and reserved by Memorial Sloan Kettering Cancer Center

Last Updated

December 12, 2022

Learn about our [Health Information Policy](#).



Memorial Sloan Kettering
Cancer Center