PATIENT & CAREGIVER EDUCATION

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.
- It is used with another drug (cipaglucosidase alfa) to treat Pompe disease.
- It may be given to you for other reasons. Talk with the doctor.

What do I need to tell my doctor BEFORE I take this drug?

- If you are allergic to this drug; any part of this drug; or any other drugs, foods, or substances. Tell your doctor

about the allergy and what signs you had.

- If you have kidney disease.
- If you are pregnant or may be pregnant. Some brands of this drug are not for use during pregnancy.
- If you are breast-feeding. Do not breast-feed while you take this drug.

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

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

What are some things I need to know or do while I take this drug?

All products:

- Tell all of your health care providers that you take this drug. This includes your doctors, nurses, pharmacists, and dentists.
- Have your blood work and other lab tests checked as you have been told by your doctor.
- This drug may cause diarrhea. If you get diarrhea, talk

with your doctor about ways to lower this effect. Do not stop taking this drug without talking with your doctor.

- This drug has caused fertility problems in animals. Fertility problems may affect being able to get pregnant or father a child.

Products used to treat Gaucher disease or Niemann-Pick type C disease:

- You may bleed more easily. Be careful and avoid injury. Use a soft toothbrush and an electric razor.
- Follow the diet plan that your 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.
- This drug may cause harm to the unborn baby if you take it while you are pregnant. If you are pregnant or you get pregnant while taking this drug, call your doctor right away.

Products used with cipaglucosidase alfa to treat Pompe disease:

- This drug is taken with another drug called cipaglucosidase alfa. Be sure you know about the warnings, benefits, and risks. Talk with the doctor if you have questions or concerns.

- This drug may cause harm to an unborn baby. A pregnancy test will be done before you start this drug to show that you are NOT pregnant.
- If you may become pregnant, use birth control while taking this drug and for 60 days after your last dose. If you get pregnant, call your doctor right away.

What are some side effects that I need to call my 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 doctor or get medical help right away if you have any of the following signs or symptoms that may be related to a very bad side effect:

All products:

- 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.

Products used to treat Gaucher disease or Niemann-Pick type C disease:

- 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.

Products used with cipaglucosidase alfa to treat Pompe disease:

- Fever.

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 doctor or get medical help if any of these side effects or any other side effects bother you or do not go away:

All products:

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

Products used to treat Gaucher disease or Niemann-Pick type C disease:

- Weight loss.
- Gas.
- 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 your doctor right away if you have new or worsening hand shakiness or tremors.

Products used with cipaglucosidase alfa to treat Pompe disease:

- Feeling tired or weak.

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

You may report side effects to your national health agency.

You may report side effects to the FDA at 1- 800-332-1088. You may also report side effects at https://www.fda.gov/medwatch.

How is this drug best taken?

Use this drug as ordered by your doctor. Read all information given to you. Follow all instructions closely.

Products used to treat Gaucher disease or Niemann-Pick type C disease:

- It is best to take this drug in between meals or as you have been told by your doctor.
- Take this drug at the same time of day.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.

Products used with cipaglucosidase alfa to treat Pompe disease:

- This drug is taken every other week as you have been told. Take it about 1 hour before your dose of cipaglucosidase alfa unless your doctor tells you otherwise.
- Swallow the capsule whole with water. You may also swallow it with an unsweetened drink like tea or coffee with no cream, sugar, or sweeteners.

- Do not eat or drink anything (other than unsweetened drinks) for at least 2 hours before or 2 hours after taking this drug.

What do I do if I miss a dose?

Products used to treat Gaucher disease or Niemann-Pick type C disease:

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

Products used with cipaglucosidase alfa to treat Pompe disease:

- Do not start your dose of cipaglucosidase alfa. Call your healthcare provider to reschedule.

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

All products:

- 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.

Products used with cipaglucosidase alfa to treat Pompe disease:

- Store in the original container to protect from light.

General drug facts

- If your symptoms or health problems do not get better or if they become worse, call your doctor.
- Do not share your drugs with others and do not take anyone else's drugs.
- 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.
- Some drugs may have another patient information leaflet. Check with your pharmacist. 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.

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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.

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