PATIENT & CAREGIVER EDUCATION

Nitisinone

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

Harliku; Nityr; Orfadin

Brand Names: Canada

MDK-Nitisinone; Orfadin

What is this drug used for?

- It is used to treat hereditary tyrosinemia type 1 (HT-1).
- It is used to treat alkaptonuria (AKU).

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.

This drug may interact with other drugs or health problems.

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?

- Tell all of your health care providers that you take this drug. This includes your doctors, nurses, pharmacists, and dentists.
- Have blood work checked as you have been told by the doctor. Talk with the doctor.
- Have your urine checked as you have been told by your doctor.
- Have an eye exam as you have been told by your doctor.
- Follow the diet plan that your doctor told you about.
- Tell your doctor if you are pregnant, plan on getting pregnant, or are breast-feeding. You will need to talk about the benefits and risks to you and the baby.

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:

- 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.
- Signs of liver problems like dark urine, tiredness, decreased appetite, upset stomach or stomach pain, light-colored stools, throwing up, or yellow skin or eyes.
- Change in eyesight, eye pain, or severe eye irritation.
- Eye is bothered by bright light.
- Feeling confused, not able to focus, or change in behavior.
- Redness, irritation, pain, or thick skin on the palms of the hands or soles of the feet.
- Low white blood cell counts have happened with this drug. This may lead to a higher chance of getting an infection. Call your doctor right away if you have signs of

infection like fever, chills, or sore throat.

- Low platelet counts have rarely happened with this drug. This may lead to a higher chance of bleeding. Call your doctor right away if you have any unexplained bruising or bleeding.

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:

Liquid (suspension):

- Headache.
- Upset stomach.
- Diarrhea.

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.

Capsules:

- Take on an empty stomach. Take 1 hour before or 2 hours after meals.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.
- Capsules may be opened and contents mixed with a small amount of water, formula, or applesauce.
- If mixed, swallow the mixed drug right away. Do not store for use at a later time.

Tablets:

- Take with or without food.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.
- If you cannot swallow this drug whole, some brands may be dissolved in room temperature water in an oral syringe. If you can swallow soft foods, the tablets can be crushed and mixed with applesauce. Do not mix with other liquids or foods. Follow how to mix as you have

been told by your doctor or read the package insert. Be sure you know whether your brand can be dissolved in water.

- Wash your hands before mixing.
- If dissolved in water, take within 24 hours of adding water to the tablets. If mixed in applesauce, take within 2 hours of mixing. Do not store for future use.

Liquid (suspension):

- If the adapter has not been put in the bottle when you get it, follow how to prepare the bottle as you have been told or read the package insert.
- Take with or without food.
- Shake well for at least 5 seconds before each use. Be sure all the particles have dissolved before you take this drug.
- Measure liquid doses carefully. Use the measuring device that comes with this drug. If there is none, ask the pharmacist for a device to measure this drug.
- Keep taking this drug as you have been told by your doctor or other health care provider, even if you feel well.

What do I do if I miss a dose?

Tablets:

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

All other products:

- Take a missed dose as soon as you think about it.
- If it is close to the time for your next dose, skip the missed dose and go back to your normal time.
- Do not take 2 doses at the same time or extra doses.

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

Capsules:

- Store some brands in a refrigerator. Do not freeze. If needed, you may store at room temperature up to 77°F (25°C) for up to 45 days. If stored at room temperature and not used within 45 days, throw this drug away.
- Store some brands at room temperature in a dry place. Do not store in the bathroom.
- Be sure you know how to store this brand. Ask your pharmacist if you have any questions.

Tablets:

- Store tablets in the original container at room temperature. Keep the cap tightly closed.
- Protect from light.
- Store in a dry place. Do not store in a bathroom.
- If you have mixed this drug with water or in applesauce, keep it at room temperature and out of sunlight.

Liquid (suspension):

- If the adapter has not been put in the bottle when you get it, store in a refrigerator until you need to prepare the bottle.
- Do not freeze.
- After the adapter has been put in the bottle, store at room temperature up to 77°F (25°C).
- Throw away any unused part 60 days after the adapter was put in the bottle. You or your pharmacist will need to write the date to throw away on the bottle label.

All products:

- 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 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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