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).
- 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.

This drug may interact with other drugs or health problems.

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.
- Have your child's blood work checked often. Talk with your child's doctor.
- Have your child's urine checked as you have been told by the doctor.
- Get your child an eye exam as you have been told by the doctor.
- Have your child follow the diet plan your child's doctor told you about.

If your child is pregnant or breast-feeding a baby:

- Talk with the doctor if your child is pregnant, becomes pregnant, or is breast-feeding a baby. You will need to talk about the benefits and risks to your child and the baby.

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.
- 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 child's doctor right away if your child has 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 child's doctor right away if your child has 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 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:

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 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.

Capsules:

- Give this drug on an empty stomach. Give at least 1 hour before or 2 hours after a meal.
- 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.
- Capsules may be opened and contents mixed with a small amount of water, formula, or applesauce.
- If mixed, have your child swallow the mixed drug right away. Do not store for use at a later time.

Tablets:

- Give this drug with or without food.
- 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.
- If your child cannot swallow this drug whole, you may dissolve it in room temperature water in an oral syringe. If your child 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 child's doctor or read the package insert.
- Wash your hands before mixing.
- If dissolved in water, give within 24 hours of adding water to the tablets. If mixed in applesauce, give 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.
- Give this drug with or without food.
- Shake well for at least 5 seconds before each use. Be sure all the particles have dissolved before giving 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 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?

Tablets:

- 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.

All other products:

- Give a missed dose as soon as you think about it.
- If it is close to the time for your child's next 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?

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 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 child's 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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