

Patient information

Nitisinone (Orfadin) 2mg Hard Capsules

Pharmacy and Clinical Chemistry

Important information – Please Read the Following Carefully.

Read all of this leaflet carefully before you start taking this medicine

- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your consultant or pharmacist.
- This medicine is for you. Never share your medicine with others. It may harm them, even if they have the same symptoms or condition as you.
- Please inform your consultant or pharmacist if any of the listed side effects become really troublesome or if you notice any side effects not mentioned in this patient information leaflet.

What nitisinone is, and what it is taken for?

Nitisinone is used to treat a rare genetic condition called alkaptonuria, also known as AKU, which is present from birth. AKU is caused by a missing enzyme which means the body cannot break down a substance called homogentisic acid (HGA). When HGA builds up in the body, this can cause the symptoms of AKU that you might suffer from.

For example:

- severe arthritis of the spine and other large joints
- heart valve damage
- stones forming in the kidneys, prostate, gall bladder and salivary glands.
- rupturing (bursting or breaking) of tendons, muscles and ligaments
- fractured bones
- hearing problems
- kidney damage.

Nitisinone stops the enzyme which makes HGA from working. This stops HGA from building up in the body and causing harm.

The body makes HGA when you eat protein, especially protein containing substances called tyrosine and phenylalanine. It is important that you follow a special diet that is low in these substances while you are taking nitisinone. If you carry on eating food and drinks which contain these substances, you could get high levels of tyrosine in the blood. This can cause problems with the eyes, skin and central nervous system.

Your consultant and/or dietitian will discuss changes to your diet with you in more detail. The dietitians will provide you with more information about specific foods to avoid and give you a diet plan to follow.

Things to check before taking nitisinone capsules.

Do not take nitisinone capsules if you are allergic to nitisinone or any of the other ingredients in nitisinone capsules. If this is the case, talk to your consultant or pharmacist before starting to take this medication.

Before using this medication, tell the consultant or pharmacist your medical history, especially if you have any eye problems (e.g. eye pain, redness or swelling, if your eyes are more sensitive to light than normal or if you have other conditions such as cataracts or corneal ulcers). You may need to have an eye examination before starting treatment.

Your consultant will advise on regular visits to monitor your liver function and blood counts during treatment with nitisinone.

Other medications

There may be other drugs that interact with nitisinone. It is important that you tell your consultant and pharmacist if you are currently taking, or have recently taken, any other medication, even if these other medication do not require a prescription, such as herbal medicines or vitamin supplements.

Keep an up to date list of your medication with you and if you start, stop or change to a new dose of any of your medication, ask your consultant, pharmacist or the doctor treating you to check for any interactions with nitisinone.

Pregnancy and breastfeeding

You should contact your consultant or pharmacist before taking nitisinone if you are pregnant, or become pregnant, or wish to breastfeed.

Performing skilled tasks (e.g. driving and operating machines):

If you experience problems with your vision whilst taking nitisinone, you should contact your consultant for advice before driving or using machinery.

How to take nitisinone capsules

Always take nitisinone tablets exactly as your doctor has told you to. You should check with your consultant or pharmacist if you are not sure.

Method of administration

Nitisinone capsules should be taken by mouth with food.

Dose

The normal dose of nitisinone is one capsule daily. Take your dose at the same time each day. In order to get the most benefit from this medication, it is important that you take it regularly.

If you have forgotten to take your nitisinone capsules

Take the missed dose as soon as you remember. If it is almost time for your next scheduled dose, skip the missed dose and take your next dose as directed.

Do not take a double dose to make up for a forgotten tablet.

What to do if you take more nitisinone capsules than you should

If you take too many tablets, or someone else takes your medicine, tell a doctor or go to your closest Emergency Department (A&E Department) straight away. Show any left-over medicines or the empty box to the doctor. You should also contact, or ask your doctor to contact, the National Alkaptonuria Centre (NAC) in Liverpool for advice.

Possible side effects of nitisinone

Like all medicines, nitisinone can cause side effects, although not everybody will get them.

Some side effects may result from not properly following your diet plan and eating or drinking foods that you are meant to avoid.

Do not worry. It is important to know that these side effects may occur, but only very rarely.

Stop taking nitisinone and see a doctor straight away, if you notice any of the following serious side effects - you may need urgent medical treatment: attend your nearest Emergency Department immediately.

- an allergic reaction (e.g. itching or hives, swelling in face or hands, swelling or tingling in the mouth or throat, tightness in chest or trouble breathing).
- black, tarry or bloody stools
- dark-colored urine or pale stools
- extreme thirst or hot, dry skin
- problems with vision, or eyes feeling more sensitive to light
- red, sore, painful, puffy, or irritated eyes
- sudden stomach pain, constipation, vomiting
- unusual bleeding, bruising, or weakness
- yellow eyes or skin

Other, less serious side effects may be more likely to occur. Continue using nitisinone and talk with your consultant or pharmacist if you have any of these side effects:

- fever, sore throat, cough and muscle aches
- itchy, flaky, or peeling skin rash
- diarrhoea, nausea, vomiting or constipation
- feeling nervous or tired
- changes in your menstrual period
- thirst
- mild stomach pains
- difficulty concentrating or memory problems.

Please tell your consultant or pharmacist if any of the side effects listed become really troublesome or if you notice any side effects not listed in this leaflet.

How to store nitisinone capsules

Keep nitisinone capsules out of the reach and sight of children.

Nitisinone capsules should be stored in the refrigerator (between 2-8°C). Keep nitisinone capsules in the original packaging and protect the contents from light.

Do not take the medicine after the expiry date printed on the label. The expiry date refers to the last day of the month. Medicines should not be put in household waste or wastewater.

If you do not use all of the capsules provided, return the unused capsules to a pharmacy.

What is in nitisinone capsules?

The active ingredient in this medicine is nitisinone. The other ingredients are pregelatinised starch (maize), gelatin, titanium dioxide (E171), black iron oxide (E172), shellac and propylene glycol.

Licensed status of the product:

Nitisinone is now licensed for use in AKU following the success of the DevelopAKUre clinical trials. Your consultant/AKU team will explain the risks and benefits before prescribing nitisinone for you.

The information contained in this leaflet applies only to nitisinone capsules for AKU. If you have any questions or you are not sure about anything, ask your consultant or pharmacist.

Who to contact if you need more information or advice

If you have any further questions, please do not hesitate to contact us. The doctors and other healthcare professionals at National Alkaptonuria Centre (NAC) have practical experience and additional information written for healthcare professionals which they can use to answer your questions.

Feedback

Your feedback is important to us and helps us influence care in the future.

Following your discharge from hospital or attendance at your outpatient appointment you will receive a text asking if you would recommend our service to others. Please take the time to text back, you will not be charged for the text and can opt out at any point. Your co-operation is greatly appreciated.

Further Information

Dr M Khedr, Consultant in Clinical Biochemistry and Metabolic Medicine, Tel :0151 706 4197

**AKU Clinical Nurse Specialists and NAC coordinator,
Tel: 0151 706 4289**

Pharmacists

**Medicines Information can be contacted on
Tel: 0151 706 2096 for advice about your medication.**

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All Trust approved information is available on request in alternative formats, including other languages, easy read, large print, audio, Braille, moon and electronically.

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