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Nityr (*nitisinone*)

An overview of Nityr and why it is authorised in the EU

What is Nityr and what is it used for?

Nityr is a medicine used to treat hereditary tyrosinaemia type 1 (HT-1). This is a rare disease in which the body is unable to completely break down the amino acid tyrosine and, as a result, harmful substances are formed, causing serious liver problems and liver cancer.

Nityr is used together with a diet that restricts the intake of the amino acids tyrosine and phenylalanine. These amino acids are normally found in proteins in foods and drinks.

Nityr contains the active substance nitisinone and is a 'generic medicine'. This means that Nityr contains the same active substance and works in the same way as a 'reference medicine' already authorised in the EU called Orfadin. For more information on generic medicines, see the question-and-answer document here.

How is Nityr used?

Nityr can only be obtained with a prescription and treatment should be started and monitored by doctors who have experience in the treatment of patients with HT-1. Treatment should be started as early as possible and the dose of Nityr adjusted according to the patient's response and body weight.

Nityr is available as 10 mg tablets to be taken by mouth. The recommended starting dose is 1 mg per kilogram body weight per day.

Nityr is intended for long-term use. Patients should be monitored at least every six months.

For more information about using Nityr, see the package leaflet or contact your doctor or pharmacist.

How does Nityr work?

Tyrosine is broken down in the body by a number of enzymes. Patients with HT-1 lack one of these enzymes, so tyrosine is not properly eliminated but is converted into harmful substances. The active substance in Nityr, nitisinone, blocks an enzyme that converts tyrosine into harmful substances. However, as the unconverted tyrosine remains in the body during Nityr treatment, patients need to eat

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a special diet low in tyrosine. The diet also needs to be low in phenylalanine, as this is converted to tyrosine in the body.

How has Nityr been studied?

Studies on the benefits and risks of the active substance in the authorised use have already been carried out with the reference medicine, Orfadin, and do not need to be repeated for Nityr.

As for every medicine, the company provided studies on the quality of Nityr. The company also carried out a study that showed that it is 'bioequivalent' to the reference medicine. Two medicines are bioequivalent when they produce the same levels of the active substance in the body and are therefore expected to have the same effect.

What are the benefits and risks of Nityr?

Because Nityr is a generic medicine and is bioequivalent to the reference medicine, its benefits and risks are taken as being the same as the reference medicine's.

Why is Nityr authorised in the EU?

The European Medicines Agency concluded that, in accordance with EU requirements, Nityr has been shown to have comparable quality and to be bioequivalent to Orfadin. Therefore, the Agency's view was that, as for Orfadin, the benefit of Nityr outweighs the identified risk and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Nityr?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Nityr have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Nityr are continuously monitored. Side effects reported with Nityr are carefully evaluated and any necessary action taken to protect patients.

Other information about Nityr

Nityr received a marketing authorisation valid throughout the EU on 26 July 2018.

Further information on Nityr can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. Information on the reference medicine can also be found on the Agency's website.

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