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EPAR summary for the public

Nitisinone MDK[1]

nitisinone

This is a summary of the European public assessment report (EPAR) for Nitisinone MDK. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Nitisinone MDK.

For practical information about using Nitisinone MDK, patients should read the package leaflet or contact their doctor or pharmacist.

What is Nitisinone MDK and what is it used for?

Nitisinone MDK 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 harmful substances are formed, causing serious liver problems and liver cancer.

Nitisinone MDK 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.

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

Nitisinone MDK contains the active substance nitisinone.

How is Nitisinone MDK used?

Nitisinone MDK 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

[1] Previously known as Nitisinone MendeliKABS

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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be started as early as possible and the dose of Nitisinone MDK adjusted according to the patient's response and body weight.

Nitisinone MDK is available as capsules (2 mg, 5 mg and 10 mg). The recommended starting dose is 1 mg per kilogram body weight per day. The capsules are usually swallowed whole, but they may be opened and their contents mixed into a small amount of water or formula just before swallowing.

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

How does Nitisinone MDK 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 transformed into harmful substances. The active substance in Nitisinone MDK, nitisinone, blocks an enzyme that converts tyrosine into harmful substances. However, as the unconverted tyrosine remains in the body during Nitisinone MDK treatment, patients need to eat a special diet low in tyrosine. The diet also needs to be low in phenylalanine, as this is converted into tyrosine in the body.

How has Nitisinone MDK been studied?

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

As for every medicine, the company provided studies on the quality of Nitisinone MDK. 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 Nitisinone MDK?

Because Nitisinone MDK 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 Nitisinone MDK approved?

The European Medicines Agency concluded that, in accordance with EU requirements, Nitisinone MDK 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 outweighs the identified risk. The Agency recommended that Nitisinone MDK be approved for use in the EU.

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

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

Other information about Nitisinone MDK

The European Commission granted a marketing authorisation valid throughout the European Union for Nitisinone MendeliKABS on 24 August 2017. The name of the medicine was changed to Nitisinone MDK on 3 October 2017.

The full EPAR for Nitisinone MDK can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Nitisinone MDK, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The full EPAR for the reference medicine can also be found on the Agency's website.

This summary was last updated in 10-2017.