22 June 2017
EMA/CHMP/368249/2017
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Nitisinone MendeliKABS

nitisinone

On 22 June 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Nitisinone MendeliKABS, intended for treatment of hereditary tyrosinemia type 1 (HT-1). The applicant for this medicinal product is MendeliKABS Europe Ltd.

Nitisinone MendeliKABS will be available as 2 mg, 5 mg and 10 mg hard capsules. The active substance of Nitisinone MendeliKABS is nitisinone (ATC code: A16AX04). It is an inhibitor of the 4-hydroxyphenyl-pyruvate dioxygenase (HDDP), an enzyme involved in tyrosine degradation. By inhibiting the normal catabolism of tyrosine in patients with HT-1, nitisinone prevents the accumulation of maleylacetoacetate and fumarylacetoacetate,which are converted into toxic metabolites.

Nitisinone MendeliKABS is a generic of Orfadin, which has been authorised in the EU since 21 February 2005. Studies have demonstrated the satisfactory quality of Nitisinone MendeliKABS, and its bioequivalence to the reference product Orfadin. A question and answer document on generic medicines can be found here.

The full indication is:

“Treatment of adult and paediatric (in any age range) patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.”

It is proposed that Nitisinone MendeliKABS be prescribed by physicians experienced in the treatment of HT-1.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

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