



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

31 May 2018  
EMA/CHMP/230825/2018  
Committee for Medicinal Products for Human Use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

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### Nityr nitisinone

On 31 May 2018, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Nityr, intended for treatment of hereditary tyrosinemia type 1 (HT-1). The applicant for this medicinal product is Cycle Pharmaceuticals Ltd.

Nityr will be available as 10 mg tablets. The active substance of Nityr is nitisinone, an inhibitor of 4-hydroxyphenyl-pyruvate dioxygenase (HDDP), an enzyme involved in tyrosine degradation (ATC code: A16AX04). 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.

Nityr is a generic of Orfadin, which has been authorised in the EU since 21 February 2005. Studies have demonstrated the satisfactory quality of Nityr, 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 patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine."

It is proposed that Nityr 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.

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<sup>1</sup> Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

