



Final Appraisal Recommendation

Advice No: 1021 – September 2021

Nitisinone (Orfadin[®]) 10 mg hard capsules

Swedish Orphan Biovitrum Ltd (Sobi Ltd)

Recommendation of AWMSG

Nitisinone (Orfadin[®]) is recommended for use within NHS Wales for the treatment of adult patients with alkaptonuria.

This recommendation applies only in circumstances where the approved Wales Patient Access Scheme (WPAS) is utilised or where the list/contract price is equivalent or lower than the WPAS price.

Additional note(s):

- AWMSG considered nitisinone (Orfadin[®]) as an ultra-orphan equivalent medicine according to the criteria in the AWMSG appraisal process for a medicine for a rare disease.
- Please refer to the Summary of Product Characteristics for the full licensed indication.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 2322), which includes the AWMSG Secretariat Assessment Report (ASAR), the Preliminary Appraisal Recommendation (PAR) and the applicant company's response to the PAR, clinical expert opinion (where available), the views of patients/patient carers (where available) and the lay member perspective.

This recommendation has been ratified by Welsh Government and will be considered for review every three years.

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