



Final Appraisal Recommendation

Advice number: 0922 – April 2022

Mercaptamine bitartrate (Procysbi®) 25 mg and 75 mg gastro-resistant hard capsules

Submission by Chiesi Limited

Recommendation of the All Wales Medicines Strategy Group

Mercaptamine bitartrate (Procysbi®) is recommended as an option for use within NHS Wales for the treatment of proven nephropathic cystinosis. Mercaptamine bitartrate (also known as cysteamine) reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure.

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 mercaptamine bitartrate (Procysbi®) as an ultra-orphan medicine according to the criteria in the AWMSG appraisal process for a medicine for a rare disease.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 4804), 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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capsules. April 2022.