Grŵp Strategaeth Meddyginiaethau Cymru Gyfan
All Wales Medicines Strategy Group

Final Appraisal Recommendation

Advice number: 2122 – October 2022

Migalastat hydrochloride (Galafold®) 123 mg hard capsules

Submission by Amicus Therapeutics UK Ltd

Recommendation of the All Wales Medicines Strategy Group

Migalastat hydrochloride (Galafold®) is recommended as an option for restricted use within NHS Wales.

Migalastat hydrochloride (Galafold®) is licensed for the long-term treatment of adolescents aged 12 years to 16 years with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation.

Migalastat hydrochloride (Galafold®) is restricted for use for the treatment of Fabry disease in adolescents aged 12 years to 16 years with an amenable mutation, only if enzyme replacement therapy (ERT) would otherwise be offered.

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

Additional note(s):

- AWMSG considered migalastat hydrochloride (Galafold®) as an ultra-orphan medicine according to the criteria in the AWMSG appraisal process for a medicine for a rare disease.
- See NICE guidance for migalastat hydrochloride (Galafold®) for the treatment of Fabry disease in adults and adolescents aged over 16 years (HST4, originally published February 2017).

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 4268), which includes the All Wales Toxicology and Therapeutics Centre (AWTTC) assessment form, 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 after three years.