1 April 2016
EMA/CHMP/220589/2016
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Galafold

migalastat

On 1 April 2016, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Galafold, intended for the treatment of Fabry disease. Galafold was designated as an orphan medicinal product on 22 May 2006. The applicant for this medicinal product is Amicus Therapeutics UK Ltd.

Galafold will be available as 123 mg hard capsules. The active substance of Galafold is migalastat, a pharmacological chaperone designed to selectively and reversibly bind to certain mutant forms of the α-Gal A enzyme, in patients with GLA mutations responsive to migalastat (defined as amenable mutations). Migalastat binding stabilizes the enzyme thus facilitating its trafficking to the lysosomes where the enzyme activity is restored.

The benefits of Galafold are its ability to stabilise renal function for up to 18 months in patients with experience of enzyme replacement therapy (ERT) and up to 30 months in ERT-naïve patients. It also improves cardiac function following 18 months of treatment in ERT-experienced patients and 30 months of treatment in ERT-naïve patients. The most common side effect is headache which is seen in more than 10% of patients.

The full indication is: "Galafold is indicated for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation."

It is proposed that Galafold is prescribed by physicians experienced in the treatment of Fabry disease.

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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