Summary of opinion\(^1\) (initial authorisation)

**Elfabrio**

**pegunigalsidase alfa**

On 23 February 2023, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Elfabrio\(^2\), intended for the treatment of Fabry disease. The applicant for this medicinal product is Chiesi Farmaceutici S.p.A.

Elfabrio will be available as a 2 mg/ml concentrate for solution for infusion. The active substance of Elfabrio is pegunigalsidase alfa, a recombinant human α-galactosidase-A (ATC code: A16AB20), which is an enzyme replacement therapy that provides an exogenous source of α-galactosidase-A.

The benefit of Elfabrio is its ability to reduce renal disease substrates of Fabry disease, which appeared to translate into reduced deterioration of renal function in adults with Fabry disease. The most common side effects are infusion-related reactions, hypersensitivity and asthenia.

The full indication is:

Elfabrio is indicated for long-term enzyme replacement therapy in adult patients with a confirmed diagnosis of Fabry disease (deficiency of alpha-galactosidase).

Elfabrio treatment must be managed by a physician experienced in the treatment of patients with 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

2 This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained