15 December 2022
EMA/CHMP/794393/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pombiliti

cipaglucosidase alfa

On 15 December 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Pombiliti[2], intended for the treatment of glycogen storage disease type II (Pompe disease). The applicant for this medicinal product is Amicus Therapeutics Europe Limited.

Pombiliti will be available as a 105 mg powder for concentrate for solution for infusion. The active substance of Pombiliti is cipaglucosidase alfa, a recombinant human acid α-glucosidase (ATC code: A16AB23), which is an enzyme replacement therapy that provides an exogenous source of acid α-glucosidase.

The benefit of Pombiliti is its ability to improve the motor function (six-minute walk distance) of patients with late-onset Pompe disease when used in combination with miglustat. The most common side effects are infusion-associated reactions such as chills, dizziness, urticaria, flushing, somnolence, anaphylaxis, chest discomfort, cough, infusion site swelling, and pain.

The full indication is:

> Pombiliti (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency).

Treatment should be supervised by a physician experienced in the management of Pompe disease or other inherited metabolic or neuromuscular diseases.

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

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union