



26 April 2023
EMA/CHMP/794403/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Opfolda miglustat

On 26 April 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 Opfolda, intended for the treatment of glycogen storage disease type II (Pompe disease) in combination with cipaglucosidase alfa. The applicant for this medicinal product is Amicus Therapeutics Europe Limited.

Opfolda will be available as a 65 mg hard capsule. The active substance of Opfolda is miglustat (ATC code: A16AX06), a pharmacokinetic enzyme stabiliser that binds selectively with cipaglucosidase alfa in the blood during infusion and minimises the loss of enzyme activity while in circulation.

The benefit of Opfolda is its ability to stabilise cipaglucosidase alfa, a recombinant human acid α -glucosidase, when co-administered in patients with late-onset Pompe disease. The most common side effect is constipation.

Opfolda is a hybrid medicine² of Zavesca which has been authorised in the EU since 2002. Opfolda contains the same active substance as Zavesca but in a lower strength. It is also authorised for a different indication and can only be used in combination with cipaglucosidase alfa.

The full indication is:

Opfolda (miglustat) is an enzyme stabiliser of cipaglucosidase alfa long-term enzyme replacement therapy in adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency).

Treatment should be supervised by a physician experienced in the management of patients with 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

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 Hybrid applications rely in part on the results of pre-clinical tests and clinical trials for a reference product and in part on new data.



granted by the European Commission.