Drugs

PEER-REVIEWED FEATURE

Cipaglucosidase Alfa: Adis Evaluation

Key Points

- A long-term ERT being developed by Amicus Therapeutics for the treatment of Pompe disease
- Received its first approval on 27 March 2023 in the EU
- Approved for use in combination with miglustat for the treatment of adults with late-onset Pompe disease

Summary

Cipaglucosidase alfa (Pombiliti™) is a recombinant human acid α-glucosidase (GAA) product being developed by Amicus Therapeutics along with the enzyme stabilizer miglustat as a two-component therapy for Pompe disease.

Pompe disease is a rare, inherited lysosomal disease caused by a deficiency of the enzyme GAA, which leads to accumulation of glycogen in various tissues.

On 27 March 2023, cipaglucosidase alfa was approved in the EU as a long-term enzyme replacement therapy (ERT) used in combination with miglustat for the treatment of adults with late-onset Pompe disease.