



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Pombiliti (*cipagluco­sidase alfa*)

An overview of Pombiliti and why it is authorised in the EU

### What is Pombiliti and what is it used for?

Pombiliti is a medicine used to treat adults with late-onset Pompe disease (acid alpha-glucosidase [GAA] deficiency), an inherited disorder in which patients have breathing difficulties and muscle weakness. Pombiliti is used in combination with another medicine, miglustat.

Pombiliti contains the active substance cipagluco­sidase alfa.

### How is Pombiliti used?

Pombiliti is given by infusion (drip) into a vein every other week. The infusion starts 1 hour after taking miglustat and lasts 4 hours. Pombiliti may be given at home in patients who tolerate their infusions well.

The medicine can only be obtained with a prescription and treatment should be supervised by a physician experienced in the management of patients with Pompe disease or similar diseases.

For more information about using Pombiliti, see the package leaflet or contact your doctor or pharmacist.

### How does Pombiliti work?

Patients with Pompe disease lack an enzyme called acid alpha-glucosidase (GAA) which is important for breaking down glycogen (a complex sugar stored in the body) into glucose (a simple sugar). As a result, glycogen accumulates in the muscles, including the heart and diaphragm (the main breathing muscle under the lungs), causing heart problems, breathing difficulties and muscle weakness.

The active substance in Pombiliti, cipagluco­sidase alfa, is an enzyme that acts in the same way as the missing GAA enzyme (i.e. breaking down glycogen into glucose). It replaces GAA and prevents further damage caused by the build-up of glycogen. Pombiliti is given in combination with miglustat, an enzyme stabilizer which helps cipagluco­sidase alfa to stay functional.

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## **What benefits of Pombiliti have been shown in studies?**

A main study involving 125 patients showed that Pombiliti in combination with miglustat improved the physical abilities of patients with late-onset Pompe disease. The study looked at changes in the distance patients were able to walk before and after treatment. On average, patients treated with Pombiliti and miglustat for 1 year were able to walk 20 more metres in 6 minutes than they could before treatment.

## **What are the risks associated with Pombiliti?**

The most common side effects with Pombiliti (which may affect up to 1 in 10 people) are chills, dizziness, flushing, sleepiness, chest discomfort, cough, swelling at the infusion site and pain.

Serious side effects (which may affect up to 1 in 50 people) are itchy rash, serious allergic reactions, fever, feeling faint, difficulty breathing, swelling in the throat, wheezing and low blood pressure.

Pombiliti must not be used in people who have had life-threatening allergic reactions to the active substance or to any of the other ingredients of the medicine. It must also not be used in people who cannot have miglustat.

For the full list of side effects and restrictions, see the package leaflet.

## **Why is Pombiliti authorised in the EU?**

Pompe disease is a rare and debilitating disease, with patients having life-threatening heart and respiratory problems. Studies show that, when given in combination with miglustat, Pombiliti is effective at improving or stabilising the physical abilities of patients with late-onset Pompe disease. The side effects of Pombiliti, which are mostly mild to moderate, are considered manageable. The European Medicines Agency therefore decided that the benefits of Pombiliti are greater than its risks and it can be authorised for use in the EU.

## **What measures are being taken to ensure the safe and effective use of Pombiliti?**

The company that markets Pombiliti will provide educational materials on home infusion to healthcare professionals who are expected to prescribe, dispense or use Pombiliti. They are intended to provide guidance on how Pombiliti and miglustat should be given to the patients and how to manage risks related to the infusion, such as allergic reactions. Patients will also be provided with a guide on home infusion and an infusion diary to help recognise and report infusion reactions.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Pombiliti and miglustat have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Pombiliti in combination with miglustat are continuously monitored. Suspected side effects reported with Pombiliti are carefully evaluated and any necessary action taken to protect patients.

## **Other information about Pombiliti**

Further information on Pombiliti can be found on the Agency's website:

[ema.europa.eu/medicines/human/EPAR/pombiliti](https://ema.europa.eu/medicines/human/EPAR/pombiliti)