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Opfolda (*miglustat*)

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

What is Opfolda and what is it used for?

Opfolda is a medicine used in the treatment of adults with late-onset Pompe disease (acid alpha-glucosidase [GAA] deficiency), an inherited disorder in which patients have breathing difficulties and muscle weakness. It is used in combination with another medicine, cipaglucosidase alfa.

Opfolda is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but it is available as capsules of a different strength.The reference medicine for Opfolda is Zavesca.

Opfolda contains the active substance miglustat.

How is Opfolda used?

Opfolda capsules are taken by mouth approximately 1 hour but no more than 3 hours before a cipaglucosidase alfa infusion (drip).

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

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

How does Opfolda work?

The active substance in Opfolda, miglustat, attaches to cipaglucosidase alfa during treatment. This makes cipaglucosidase alfa more stable, so it can continue to be absorbed from the blood by the muscle cells that are affected by Pompe disease.

What benefits of Opfolda have been shown in studies?

One main study in 125 adults with Pompe disease compared the effects of Opfolda plus cipaglucosidase alfa with alglucosidase alfa (another medicine for Pompe disease) plus placebo (a dummy treatment).

The study showed that, after one year of treatment, the distance patients could walk in six minutes improved by around 20 meters in patients treated with Opfolda plus cipaglucosidase alfa, compared

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with an improvement of around 8 meters in patients on alglucosidase alfa and placebo. This difference was considered clinically relevant.

What are the risks associated with Opfolda?

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

The most common side effect with Opfolda (which may affect up to 1 in 10 people) is constipation.

Opfolda must not be used by people who cannot take cipaglucosidase alfa.

Why is Opfolda authorised in the EU?

The European Medicines Agency concluded that the effects of Opfolda in combination with cipaglucosidase alfa are clinically relevant and that their use offers an alternative treatment option for adult patients with late-onset Pompe disease. The safety profile of the combination is considered acceptable and comparable to that of alglucosidase alfa.

The Agency therefore decided that Opfolda's benefits 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 Opfolda?

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

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

Other information about Opfolda

Opfolda received a marketing authorisation valid throughout the EU on 26 June 2023.

Further information on Opfolda can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/opfolda.

This overview was last updated in 06-2023.