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Orladeyo (*berotralstat*)

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

What is Orladeyo and what is it used for?

Orladeyo is a medicine used to prevent attacks of hereditary angioedema (swelling) in patients from 12 years of age. Patients with hereditary angioedema have attacks of rapid swelling such as in the face, throat, arms and legs, or around the gut.

Hereditary angioedema is rare, and Orladeyo was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 June 2018. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3182028.

Orladeyo contains the active substance berotralstat.

How is Orladeyo used?

Orladeyo is available as capsules and can only be obtained with a prescription. The recommended dose is one 150-mg capsule daily taken with food.

For more information about using Orladeyo, see the package leaflet or contact your healthcare provider.

How does Orladeyo work?

The active substance in Orladeyo, berotralstat, works by blocking the activity of a protein called kallikrein. In patients with angioedema, overactive kallikrein leads to raised levels of another protein, bradykinin. Bradykinin is involved in a process that causes blood vessels to widen and become leaky. resulting in the swelling and inflammation of angioedema attacks. Blocking the activity of kallikrein reduces the number of these attacks.

What benefits of Orladeyo have been shown in studies?

Orladeyo was effective at preventing attacks of angioedema in one main study of 121 patients with hereditary angioedema. Patients taking 150 mg Orladeyo every day for 24 weeks had an average of 1.3 attacks per month, compared with 2.4 attacks per month in patients taking placebo (a dummy treatment).

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What are the risks associated with Orladeyo?

The most common side effects with Orladeyo (which may affect more than 1 in 10 people) are headache, abdominal (belly) pain and diarrhoea. Abdominal pain and diarrhoea, which can occur about 1 to 3 months after starting Orladeyo, get better as treatment continues.

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

Why is Orladeyo authorised in the EU?

Orladeyo is effective at reducing the number of angioedema attacks, including life-threatening throat swelling. The medicine can be taken by mouth and its side effects were manageable. The European Medicines Agency therefore decided that Orladeyo'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 Orladeyo?

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

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

Other information about Orladeyo

Orladeyo received a marketing authorisation valid throughout the EU on 30 April 2021.

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

This overview was last updated in 04-2021.