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Isturisa (*osilodrostat*)

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

What is Isturisa and what is it used for?

Isturisa is a medicine used to treat adults with Cushing's syndrome, a disease characterised by an excess production of the hormone cortisol by the adrenal glands, two glands situated above the kidneys.

Cushing's syndrome is rare, and Isturisa was designated an 'orphan medicine' (a medicine used in rare diseases) on 15 October 2014. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3141345.

Isturisa contains the active substance osilodrostat.

How is Isturisa used?

The medicine can only be obtained with a prescription, and treatment should be started and supervised by a doctor experienced in endocrinology or internal medicine and with access to the appropriate facilities for assessing the patient's response to Isturisa.

Isturisa is available as tablets (1, 5 and 10 mg), and the recommended starting dose is 2 mg twice daily. Patients of Asian ancestry should start Isturisa at a dose of 1 mg twice daily. The dose can be gradually increased according to the levels of cortisol in the body, which is measured by regular checks of the urine or blood, up to a maximum dose of 30 mg twice daily. The dose should be reduced or treatment stopped temporarily if the patient experiences certain side effects. For more information about using Isturisa, see the package leaflet or contact your doctor or pharmacist.

How does Isturisa work?

The active substance in Isturisa, osilodrostat, blocks the activity of an enzyme involved in the production of cortisol called 11-beta-hydroxylase. This reduces cortisol production and cortisol levels in the body, thereby relieving the symptoms of the disease.

What benefits of Isturisa have been shown in studies?

Isturisa was shown to be effective at lowering the levels of cortisol in one main study involving 137 patients with Cushing's syndrome. All patients were initially treated with Isturisa for 26 weeks. The

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dose was adjusted for each patient until their levels of cortisol were under control and within the normal range.

After this initial phase, patients whose cortisol levels were under control (71 patients) were given either Isturisa or placebo (a dummy treatment), and the study looked at the number of patients whose cortisol levels remained under control. After 8 weeks of treatment, 86% (31 out of 36) of patients treated with Isturisa had their cortisol levels under control, compared with 29% (10 out of 34) of patients given placebo.

What are the risks associated with Isturisa?

The most common side effects with Isturisa (which may affect more than 1 in 10 people) are adrenal insufficiency (low levels of cortisol produced by the adrenal glands), tiredness, nausea (feeling sick), headache, vomiting and oedema (swelling).

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

Why is Isturisa authorised in the EU?

Isturisa is effective at reducing elevated cortisol levels in patients with Cushing's syndrome. Side effects are considered manageable by adjusting the dose or temporarily stopping treatment. The European Medicines Agency therefore decided that Isturisa'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 Isturisa?

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

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

Other information about Isturisa

Isturisa received a marketing authorisation valid throughout the EU on 9 January 2020.

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

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