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Pyrukynd (*mitapivat*)

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

What is Pyrukynd and what is it used for?

Pyrukynd is a medicine used to treat adults with pyruvate kinase deficiency (PKD), an inherited disease that causes red blood cells to break down faster than normal.

PKD is rare, and Pyrukynd was designated an 'orphan medicine' (a medicine used in rare diseases) on 22 April 2020. Further information on the orphan designation can be found here: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu-3-20-2270.

Pyrukynd contains the active substance mitapivat.

How is Pyrukynd used?

Pyrukynd can only be obtained with a prescription. It is available as tablets to be taken by mouth. The recommended starting dose is one 5 mg tablet taken twice a day. The dose can be increased every four weeks, based on the patient's haemoglobin (the protein in red blood cells that carries oxygen around the body) levels and their need for a transfusion in the previous 8 weeks. The maximum recommended dose of Pyrukynd is 50 mg twice a day.

If treatment needs to be interrupted or stopped completely, the dose of Pyrukynd should be gradually reduced over a period of 1 to 2 weeks.

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

How does Pyrukynd work?

Patients with PKD have a defective form of pyruvate kinase, a protein in red blood cells which converts glucose into energy. As a result, their red blood cells cannot make enough energy to hold their shape, causing them to break down before the body has time to replace them. This excessive breakdown of red blood cells is known as haemolytic anaemia.

The active substance in Pyrukynd, mitapivat, attaches to and activates pyruvate kinase, causing it to work more effectively and thereby preventing the red blood cells of these patients from being broken down too fast.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

What benefits of Pyrukynd have been shown in studies?

The benefits of Pyrukynd were evaluated in two main studies. In the first study, involving 80 patients with PKD who were not regularly receiving blood transfusions, Pyrukynd was compared with placebo (dummy treatment). In this study, 40% of patients treated with Pyrukynd had an increase of their haemoglobin levels of 1.5 g/dL, which was maintained at 2 or more check-ups carried out after 16, 20 and 24 weeks of treatment, compared with none of the patients treated with placebo.

In the second study, involving 27 patients who were regularly receiving blood transfusions, Pyrukynd was not compared with placebo or any other medicines. In this study, the volume of red blood cells received in transfusions was reduced by more than a third in 37% of patients.

What are the risks associated with Pyrukynd?

The most common side effects with Pyrukynd (which may affect more than 1 in 10 people) are insomnia (difficulty sleeping), nausea (feeling sick) and decreased levels of the hormone oestrone seen in blood tests in male patients.

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

Why is Pyrukynd authorised in the EU?

There are limited treatment options for patients with PKD as management of the disease is restricted to supportive treatments to improve the symptoms and complications associated with haemolytic anaemia. Although there were some limitations associated with the main studies, Pyrukynd has been shown to provide clinically meaningful benefits to some patients with PKD, by increasing haemoglobin levels and reducing the need for transfusions. It was therefore considered that Pyrukynd addressed an unmet medical need in these patients.

Furthermore, the side effects of Pyrukynd are considered manageable. The European Medicines Agency therefore decided that Pyrukynd'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 Pyrukynd?

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

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

Other information about Pyrukynd

Pyrukynd received a marketing authorisation valid throughout the EU on 9 November 2022.

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

This overview was last updated in 11-2022.