EMA/471777/2017
EMEA/H/C/003937

EPAR summary for the public

Xermelo

telotristat

This is a summary of the European public assessment report (EPAR) for Xermelo. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Xermelo.

For practical information about using Xermelo, patients should read the package leaflet or contact their doctor or pharmacist.

What is Xermelo and what is it used for?

Xermelo is a medicine used to treat adults with severe diarrhoea associated with a condition called carcinoid syndrome. Carcinoid syndrome occurs when certain tumours produce and release too much of a substance called serotonin into the blood. The excess serotonin can cause severe diarrhoea as well as other symptoms such as flushing of the face and cramps. Xermelo is used together with other medicines, called somatostatin analogues, when those medicines are not sufficient on their own to control the diarrhoea.

Because the number of patients with carcinoid syndrome is low, the disease is considered 'rare', and Xermelo was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 October 2009.

Xermelo contains the active substance telotristat.

How is Xermelo used?

Xermelo can only be obtained with a prescription. It is available as tablets containing 250 mg of telotristat and the recommended dose is one tablet three times a day. Doses may need to be reduced in patients with mildly or moderately reduced liver function. Doctors may consider stopping treatment if patients do not benefit after 12 weeks. For further information, see the package leaflet.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

How does Xermelo work?

The active substance in Xermelo, telotristat, blocks the action of enzymes called L-tryptophan hydroxylases. These enzymes are needed for the production of serotonin. By blocking the enzymes, telotristat reduces the production of serotonin in patients with carcinoid syndrome, and so relieves the symptoms of the condition.

What benefits of Xermelo have been shown in studies?

The benefits of adding Xermelo to treatment have been shown in one main study involving 135 patients with carcinoid syndrome whose diarrhoea had not been fully controlled with somatostatin analogues alone. Xermelo was compared with placebo (a dummy treatment) and the main measure of effectiveness was the change in average number of daily bowel movements over the 12 weeks of the study.

At the start, patients given Xermelo had an average of 6.1 bowel movements per day, and this went down over the 12 weeks to an average of 4.7 per day, a reduction of 1.4 movements. Patients taking placebo were having an average of 5.2 bowel movements daily at the start, which fell to an average of 4.6 movements daily, a fall of 0.6 movements. The effects of Xermelo were seen from about 3 weeks after starting treatment.

What are the risks associated with Xermelo?

The most common side effects with Xermelo (which may affect more than 1 in 10 people) are abdominal (belly) pain, increases in the liver enzyme gamma-glutamyl transferase, and tiredness, and are usually mild or moderate. The commonest side effect severe enough to cause treatment to be stopped is abdominal pain.

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

Why is Xermelo approved?

Xermelo has been shown to produce a reduction in the number of bowel movements suffered by patients with carcinoid syndrome not fully controlled with other medicines. Although small, this reduction was considered significant given that patients view the frequency of bowel movements as a symptom with an important impact on their quality of life. The side effects that were seen, which mainly affected the gut, did not raise major concerns and were considered mild and manageable. The European Medicines Agency therefore decided that Xermelo's benefits are greater than its risks and recommended that it be approved for use in the EU.

What measures are being taken to ensure the safe and effective use of Xermelo?

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

Other information about Xermelo

The European Commission granted a marketing authorisation valid throughout the European Union for Xermelo on 18 September 2017.

The full EPAR for Xermelo can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Xermelo, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Xermelo can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 09-2017.