EMA/670241/2013
EMEA/H/C/002697

EPAR summary for the public

Opsumit

macitentan

This is a summary of the European public assessment report (EPAR) for Opsumit. 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 Opsumit.

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

What is Opsumit and what is it used for?

Opsumit is a medicine that contains the active substance macitentan. It is used for the long-term treatment of pulmonary arterial hypertension (PAH), a condition in which there is abnormally high blood pressure in the arteries of the lungs, causing symptoms such as breathlessness and fatigue.

Opsumit is used for adults whose PAH is classified as 'WHO functional class II to class III'. The 'class' reflects the seriousness of the disease: patients with class II PAH have slight limitation of physical activity and those with class III disease have marked limitation of physical activity. Opsumit can be used alone or in combination with other PAH medicines; for further information, see the package leaflet.

Because the number of patients with PAH is low, the disease is considered 'rare', and Opsumit was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 September 2011.

How is Opsumit used?

Opsumit can only be obtained with a prescription and treatment should be started and monitored by a doctor experienced in treating PAH. The medicine is available as 10 mg tablets and taken at a dose of one tablet every day.

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How does Opsumit work?

In PAH there is severe narrowing of the arteries of the lungs. Because more pressure is needed to force blood through the narrowed artery, this leads to high blood pressure in the lungs.

The active substance in Opsumit, macitentan, works by blocking 'endothelin receptors'. These are part of a natural mechanism in the body that can cause arteries to narrow. In patients with PAH, this mechanism is overactive and, by blocking the receptors, macitentan helps widen the arteries in the lungs and thereby bring down the blood pressure.

What benefits of Opsumit have been shown in studies?

In a main study involving 742 patients, Opsumit has been shown to reduce the risk of PAH-related illness, particularly the worsening of PAH symptoms. Patients in the study received either Opsumit or placebo (a dummy treatment) in addition to other PAH treatments for an average of 2 years. Around 37% of patients taking placebo had a worsening of their PAH symptoms compared with 24% of those who took Opsumit 10 mg.

What are the risks associated with Opsumit?

The most common side effects with Opsumit (which may affect more than 1 in 10 people) are nasopharyngitis (inflammation of the nose and throat), bronchitis (inflammation of the airways in the lungs), anaemia (low red blood cell counts) and headache. Most side effects are mild to moderate in severity. For the full list of all side effects reported with Opsumit, see the package leaflet.

In animal studies, Opsumit was shown to have an adverse effect on the development of embryos. Opsumit must therefore not be used in pregnant or breastfeeding women or in women who could become pregnant and who are not using reliable contraception. Women should also not become pregnant for one month after stopping treatment.

It must also not be used in patients with severe reduction in liver function or high levels of liver enzymes in the blood. For the full list of restrictions, see the package leaflet.

Why is Opsumit approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) concluded that Opsumit's benefits are greater than its risks and recommended that it be approved for use in the EU. Opsumit had been shown to be effective in reducing illness or deaths due to PAH and the side effects reported were similar to those reported with other medicines of its class and were considered to be manageable. However, as animal studies showed an adverse effect on the development of embryos, Opsumit must never be used in pregnant women or women who could become pregnant and are not using reliable contraception.

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

A risk management plan has been developed to ensure that Opsumit is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Opsumit, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Opsumit will send educational material to patients and healthcare professionals with information on the precautions to be taken when using Opsumit. Patients'

reminder cards will warn that the medicine must never be used in pregnant women and that women who could become pregnant must be using reliable contraception and should undergo monthly pregnancy tests.

Other information about Opsumit

The European Commission granted a marketing authorisation valid throughout the European Union for Opsumit on 20.12.2013.

The full EPAR for Opsumit 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 Opsumit, 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 Opsumit can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 12-2013.