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Opsumit (*macitentan*)

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

What is Opsumit and what is it used for?

Opsumit is a medicine 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, adolescents and children aged 2 years and older in whom PAH comes with moderate or marked limitations in physical activity (corresponding to WHO functional class II or III, respectively). Opsumit can be used alone or in combination with other PAH medicines.

Opsumit contains the active substance macitentan.

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 tablets to be taken once a day. For children, Opsumit is also available as dispersible tablets.

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

How does Opsumit work?

In PAH there is severe narrowing of the small blood vessels (arterioles) in the lungs. Because more pressure is needed to force blood through these narrowed vessels, this leads to high blood pressure in the arteries to 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 the small blood vessels to narrow. In patients with PAH, this mechanism is overactive and, by blocking these receptors, macitentan helps widen the arterioles in the lungs and thereby bring down the blood pressure.

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What benefits of Opsumit have been shown in studies?

In a main study involving 742 adults with PAH, Opsumit was shown to reduce the risk of PAH symptoms worsening. 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.

In another main study, 148 adolescents and children over 2 years of age with PAH were given Opsumit or standard treatment for an average of about 3 years. During the study, about 29% of patients given Opsumit and 32% of patients given standard treatment experienced a worsening of their disease. Data also showed that children and adolescents given Opsumit were able to achieve levels of the medicine in their blood comparable to those seen in adults.

What are the risks associated with Opsumit?

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

The most common side effects with Opsumit in adults and adolescents (which may affect more than 1 in 10 people) include nasopharyngitis (inflammation of the nose and throat), anaemia (low levels of red blood cells) and headache. Side effects in children and adolescents are comparable with those seen in adults, but also include upper respiratory tract (nose and throat) infection, rhinitis (stuffy and runny nose) and gastroenteritis (diarrhoea and vomiting).

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 in the first month after stopping treatment.

Opsumit must also not be used in patients with severe reduction in liver function or high levels of liver enzymes in the blood.

Why is Opsumit authorised in the EU?

The European Medicines Agency decided that Opsumit's benefits are greater than its risks and it can be authorised for use in the EU. In adults, Opsumit has been shown to be effective in reducing illness or deaths due to PAH. In adolescents and children, study data do not allow firm conclusions to be drawn on the benefits of Opsumit on disease progression; however, Opsumit is expected to work in the same way as it does in adults as supported by data on how the medicine behaves in the body. The side effects reported are similar to those reported with other medicines of its class and are considered to be manageable. However, 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?

The company that markets Opsumit has developed educational material for patients and healthcare professionals with information on the precautions to be taken when using Opsumit. Patients' reminder cards will include a warning 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.

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

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

Other information about Opsumit

Opsumit received a marketing authorisation valid throughout the EU on 20 December 2013.

Further information on Opsumit can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/opsumit.

This overview was last updated in 09-2024.