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Trepulmix (*treprostinil*)

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

What is Trepulmix and what is it used for?

Trepulmix is a medicine for use in the treatment of chronic thromboembolic pulmonary hypertension (CTEPH), a condition linked with high blood pressure in the lungs caused by blood clots. It can be used to improve the capacity for physical activity in patients:

- who cannot have an operation for treating the condition;
- whose condition remains or continues to return after an operation to treat it.

CTEPH is rare, and Trepulmix was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 February 2013. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3131103.

Trepulmix is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but it is used for treating a different form of pulmonary hypertension. The reference medicine for Trepulmix is Remodulin.

Trepulmix contains the active substance treprostinil.

How is Trepulmix used?

Trepulmix is given by infusion (drip) under the skin using a pump to control the speed of infusion. The dose is calculated on the basis of the patient's weight and is adjusted according to how well the condition is controlled and the severity of any side effects. The patient will be trained on using the pump and on infusing the medicine.

The medicine can only be obtained with a prescription and treatment with Trepulmix should be started and monitored only by healthcare professionals experienced in treating pulmonary hypertension. Treatment should be started in a setting where intensive care facility is available.

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

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How does Trepulmix work?

Treprostinil, the active substance in Trepulmix, works in a similar way to prostacyclin, a natural substance that widens blood vessels and stops platelets (blood components) from sticking to each other to form blood clots. In patients with CTEPH, these effects of treprostinil prevent blood clots and lower blood pressure in the pulmonary artery and so improve symptoms of the disease.

What benefits of Trepulmix have been shown in studies?

A main study involving 105 patients with severe CTEPH who could not have an operation found that Trepulmix can improve patients' capacity for physical activity, measured as the ability to walk.

In this study, patients received Trepulmix at a high dose (30 nanograms/kg/minute) or low dose (3 nanograms/kg/minute) which was not expected to have an effect. After 24 weeks, patients receiving the high dose were able to walk an average of 45 metres further in 6 minutes than at the start of treatment, compared with 4 metres further for those receiving the low dose.

Additional studies including comparison with records of patients with CTEPH who had not been treated with Trepulmix confirmed improvement in physical capacity.

What are the risks associated with Trepulmix?

The most common side effects with Trepulmix (which may affect more than 1 in 10 people) are headache, widening of blood vessels (with flushing), diarrhoea, nausea (feeling sick), jaw pain, reactions around the injection site such as pain, swelling and bleeding.

Trepulmix must not be used in patients with conditions like heart problems, stroke, problems in the gut such as ulcers, blocked veins in lungs and severe liver problems. Patients must not take Trepulmix at the same time as similar medicines called prostanoids.

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

Why is Trepulmix authorised in the EU?

The main study found that Trepulmix increases the distance patients can walk in 6 minutes. Although this study was small (because of the rarity of the disease), the results were meaningful. Additional studies showed that Trepulmix improved blood circulation and patients' physical capability.

Side effects are those expected from the way Trepulmix works and are manageable. The European Medicines Agency therefore decided that Trepulmix'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 Trepulmix?

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

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

Other information about Trepulmix

Trepulmix received a marketing authorisation valid throughout the EU on 3 April 2020.

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

This overview was last updated in 04-2020.