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Withdrawal of application for the marketing authorisation of Orepaxam (treprostinil diolamine)

Ferrer Internacional S.A. withdrew its application for a marketing authorisation of Orepaxam for the treatment of pulmonary arterial hypertension.

The company withdrew the application on 11 October 2022.

What is Orepaxam and what was it intended to be used for?

Orepaxam was developed as a medicine for the treatment of pulmonary arterial hypertension (PAH), a rare blood vessel disorder of the lungs in which the pressure in the pulmonary artery (the vessel that leads blood from the heart to the lungs) rises above normal levels. The medicine contains the active substance treprostinil diolamine.

Orepaxam was developed as a 'hybrid medicine'. This means that it is similar to a 'reference medicine' called Remodulin but there are some differences between the 2 products. Both Remodulin and Orepaxam contain treprostinil but the medicines contain a different salt (diolamine for Orepaxam and sodium for Remodulin). Orepaxam was to be available as prolonged-release tablets to be taken by mouth, while Remodulin is given by injection under the skin or infusion (drip) into a vein.

Orepaxam was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 August 2005 for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/en/medicines/human/orphan-designations/eu-3-05-310

How does Orepaxam work?

Treprostinil 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. These effects lower blood pressure in the pulmonary artery and so improve symptoms of PAH.

What did the company present to support its application?

The company provided results of four main studies that investigated the effects of Orepaxam when used on its own, or together with other therapies for PAH taken by mouth, in around 1,700 patients. In

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
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all the studies, Orepaxam was compared with placebo (a dummy treatment). Three studies looked at changes in exercise capacity measured as the improvement in the distance patients could walk in 6 minutes after 12 or 16 weeks of treatment. The fourth study looked at a delay in disease progression measured by how long it took before symptoms worsened.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn while the European Medicines Agency was still evaluating the initial information from the company.

What did the Agency recommend at that time?

As the Agency was still evaluating the initial information from the company, it had not yet made any recommendations.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that it withdrew its application because it was expected that further clinical evidence would be requested, which could not be adequately addressed with the anticipated timeframe.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no clinical trials ongoing.

There are no consequences for patients in compassionate use programmes using Orepaxam.

If you are in a compassionate use programme and need more information about your treatment, speak with your doctor.