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EPAR summary for the public

Raxone

idebenone

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

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

What is Raxone and what is it used for?

Raxone is a medicine used to treat visual impairment in adults and adolescents aged 12 years and over with Leber's hereditary optic neuropathy (LHON), an inherited disease characterised by progressive loss of sight. Raxone contains the active substance idebenone.

Because the number of patients with Leber's hereditary optic neuropathy is low, the disease is considered 'rare', and Raxone was designated an 'orphan medicine' (a medicine used in rare diseases) on 15 February 2007.

Raxone is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but Raxone contains idebenone at a different strength. The reference medicine for Raxone is Mnesis (45 mg tablets).

How is Raxone used?

Raxone can only be obtained with a prescription and treatment should be started and supervised by a doctor with experience in LHON. Raxone is available as 150 mg tablets, and the recommended dose is two tablets taken three times a day with food.

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How does Raxone work?

The active substance in Raxone, idebenone, is an anti-oxidant agent that acts on mitochondria (the structures inside cells that produce the energy necessary for cells to function). Patients affected by LHON have mutations (defects) in the genetic material of mitochondria. This means that mitochondria do not work properly to generate energy, and produce toxic forms of oxygen (free radicals) that damage nerve cells in the eye that are needed for vision. Idebenone is thought to help improve production of energy by restoring mitochondrial function, thereby preventing the cellular damage and the loss of sight seen in LHON.

What benefits of Raxone have been shown in studies?

Raxone has been investigated in one main study involving 85 patients with LHON, in which it was compared with placebo (a dummy treatment) over 24 weeks. The main measure of effectiveness was improvement in vision, mostly based on the numbers of letters patients were able to read on a standard eye test chart. By the end of the study, patients treated with Raxone were able to read on average 3 to 6 letters more compared with patients receiving placebo. Furthermore, some patients who were classified as 'off chart' (unable to read any letters on the chart) at the beginning of the study were able to read at least one line during the eye test after treatment, and this was also considered clinically important. Additionally, 30% of patients treated with Raxone (16 out of 53) had a clinically relevant recovery of vision in at least one eye, compared with 10% of patients (3 out of 29) in the placebo group.

Additional supportive data on the benefits of Raxone came from an expanded access program through which Raxone was made available to individual patients not participating in a clinical study, and from a case record survey, which included data from patients with LHON who did not receive any treatment.

Analyses of all these data showed a consistent pattern whereby generally a larger proportion of patients treated with Raxone had vision improvement compared with untreated or placebo-treated patients.

What are the risks associated with Raxone?

The most common side effects with Raxone (which may affect more than 1 in 10 people) are nasopharyngitis and cough; mild to moderate diarrhoea and back pain are also common (affecting up to 1 in 10 people).

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

Why is Raxone approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that the benefits of Raxone are greater than its risks and recommended that it be approved for use in the EU.

The Committee noted the lack of treatments for preventing or reversing the vision loss in patients with LHON. The results of the main study showed an improvement in vision in patients treated with Raxone, and this trend towards a beneficial effect was confirmed by additional data from an expanded access program and a case record survey. With regard to the safety of Raxone, the majority of side effects seen with the medicine were mild or moderate in intensity.

Raxone has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Raxone due to the rarity of the disease. Every year, the

European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

What information is still awaited for Raxone?

Since Raxone has been approved under exceptional circumstances, the company that markets Raxone will conduct additional studies on the long-term effects and safety of Raxone, and will establish and maintain a registry of LHON patients treated with Raxone.

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

A risk management plan has been developed to ensure that Raxone 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 Raxone, including the appropriate precautions to be followed by healthcare professionals and patients.

Further information can be found in the summary of the risk management plan.

Other information about Raxone

The European Commission granted a marketing authorisation valid throughout the European Union for Raxone on 8 September 2015.

The full EPAR and risk management plan summary for Raxone 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 Raxone, 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 Raxone can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 08-2015.