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Public Summary SwissPAR dated 24.07.2024

Raxone® (active substance: idebenone)

Authorisation in Switzerland: 1 March 2024

Film-coated tablets for the treatment of visual impairments with Leber's hereditary optic neuropathy

About the medicinal product

Raxone, containing the active substance idebenone, is authorised for the treatment of visual impairments in adolescents aged 12 years and older and adults with Leber's hereditary optic neuropathy (LHON). This inherited genetic defect is very rare and affects men more often than women. The condition leads to a dysfunction of the optic nerves, associated with reduced visual acuity up to blindness.

Since this is a rare disease, the medicine has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

LHON patients have a genetic defect that has a negative effect on the function of mitochondria (cell organelles that act as the powerhouse of cells). This genetic defect results in a lack of energy in the affected cells as well as an accumulation of toxic oxygen products (free radicals), leading to oxidative stress in the cell. The optic nerve cells are particularly sensitive to oxidative stress. The active substance idebenone improves energy metabolism and reduces oxidative stress in the affected cells. It acts as an antioxidant and prevents further cell damage.

Administration

Raxone is a prescription-only medicine.

It is available as a film-coated tablet in the dosage strength of 150 mg. The recommended dose of idebenone is 900 mg per day (2 × 150 mg film-coated tablets 3 times a day). The film-coated tablets are taken whole, unchewed with food.

When taking Raxone, the urine may be a reddish-brown colour. This discolouration is harmless and does not require any change to the treatment. However, the treating physician should be informed.

Efficacy

The efficacy of Raxone for LHON was investigated in 2 studies. In the RHODOS trial, 53 people aged 14 years and older who had had the disease for a maximum of 5 years were treated with Raxone for 24 months. In the control group, 28 people were given a dummy drug (placebo). The primary endpoint[1] of the trial was the *best recovery of visual acuity*. While there was a trend in favour of the Raxone group compared to the placebo group, this was not statistically significant. By contrast, trial participants treated with Raxone achieved significantly better results than those treated with placebo for the first secondary endpoint *change in best visual acuity*.

The open-label, non-placebo-controlled LEROS trial investigated the effect of Raxone in 198 patients aged 12 years and older who had had the disease for a maximum of 5 years. The results confirmed the efficacy of Raxone in the promotion of recovery and prevention of loss of visual acuity in LHON patients who had had the disease for a maximum of 5 years.

No efficacy data are available for a treatment duration of more than 24 months.

Precautions, undesirable effects, & risks

Raxone must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most frequent undesirable effects are headache in 12% of patients and rhinopharyngitis (inflammation of the mucous membranes of the nose and throat) in 11%. Liver enzyme levels may also be elevated, and this may be serious.

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

Both the RHODOS and LEROS efficacy studies demonstrated positive effects for LHON patients aged 12 years and older whose disease symptoms in the last eye developed no more than 5 years ago and for a treatment duration of a maximum of 24 months. Taking all the risks and precautions into account, and based on the available data, the benefits of Raxone outweigh the risks. Swissmedic has therefore authorised the medicinal product Raxone, containing the active substance idebenone, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: Information for health professionals Raxone®

Information for patients (package leaflet): Information for patients Raxone®

Healthcare professionals can answer any further questions.

[1] Primary efficacy endpoint: The primary endpoint is the main objective of the trial determined before the trial starts. If the primary endpoint is reached or exceeded, the trial proves that a treatment is effective. Secondary endpoints, on the other hand, refer to other effects that do not clearly prove efficacy or that do not allow any clear conclusions to be drawn about the actual target criterion (primary endpoint).

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