25 June 2015
EMA/CHMP/368035/2015
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Raxone

idebenone

On 25 June 2015, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Raxone, intended for the treatment of visual impairment in patients with Leber's Hereditary Optic Neuropathy (LHON). Raxone was designated an orphan medicinal product on 15 February 2007. The applicant for this medicinal product is Santhera Pharmaceuticals (Deutschland) GmbH.

Raxone will be available as 150 mg film-coated tablets. The active substance of Raxone is idebenone, an anti-oxidant thought to help restore mitochondrial function and prevent oxidative damage in retinal ganglion cells in LHON patients. As a result, the medicine prevents and/or reverses loss of vision in these patients.

The benefits with Raxone are its ability to improve vision in LHON patients. The most common side effects are nasopharyngitis, cough, diarrhoea and back pain.

Raxone is a hybrid of Mnesis (45mg tablets), a medicine containing the same active substance that has been authorised in Italy since 1993 but for a different indication (treatment of cognitive and behavioural deficits due to cerebral pathologies of vascular or degenerative origin).

The full indication is: "treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy".

It is proposed that treatment with Raxone be initiated and supervised by a physician with experience in LHON.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.

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made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.