



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Recommendation for maintenance of orphan designation at the time of marketing authorisation

Raxone (idebenone) for the treatment of Leber's hereditary optic neuropathy

During its meeting of 14 to 16 July 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/434 for Raxone (idebenone) as an orphan medicinal product for the treatment of Leber's hereditary optic neuropathy. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. The COMP recommended that the orphan designation of the medicine be maintained¹.

Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Raxone for:

'treatment of visual impairment in adolescent and adult patients with Leber's hereditary optic neuropathy (LHON)'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of Leber's hereditary optic neuropathy'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Leber's hereditary optic neuropathy remains a condition that is debilitating in the long term due to progressive loss of vision that may lead to blindness.

Prevalence of the condition

The sponsor provided updated information on the prevalence of Leber's hereditary optic neuropathy based on data from the scientific literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of Leber's hereditary optic neuropathy remains below the ceiling for

¹ The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with a comparable therapeutic indication cannot be placed on the market.



orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was estimated to be not more than 0.2 people in 10,000. This is equivalent to a total of not more than 10,000 people in the EU.

Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no treatments were authorised in the EU for patients affected by this condition. Patients usually received genetic counselling, general support and regular medical follow up.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Raxone still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Raxone can be found in the European public assessment report (EPAR) on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports](http://ema.europa.eu/Find%20medicine/Human%20medicines/European%20Public%20Assessment%20Reports).