



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 April 2016
EMA/COMP/156871/2016
Committee for Orphan Medicinal Products

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

Wakix (pitolisant) for the treatment of narcolepsy

During its meeting of 16 to 18 February 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/459 for Wakix (pitolisant¹) as an orphan medicinal product for the treatment of narcolepsy. 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. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with narcolepsy. The COMP recommended that the orphan designation of the medicine be maintained².

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Wakix for the treatment of narcolepsy with or without cataplexy in adults.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of narcolepsy'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Narcolepsy remains a condition that is long-term debilitating because it causes excessive daytime sleepiness and cataplexy (sudden muscle weakness), increasing the risk of accidents and interfering with normal life.

Prevalence of the condition

The sponsor provided updated information on the prevalence of narcolepsy based on data from the published literature.

¹ Previously known as 1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride

² 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.



On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of narcolepsy remains below the ceiling for 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 approximately 4 people in 10,000. This is equivalent to a total of around 205,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, modafinil and sodium oxybate were authorised in the EU for the treatment of narcolepsy.

Significant benefit of Wakix

The COMP concluded that the claim of a significant benefit of Wakix over modafinil is justified because Wakix can be tolerated better by patients and has been shown to have a positive effect on cataplexy.

When compared with sodium oxybate, Wakix was considered to improve patient care because it is to be taken by mouth once a day, whereas sodium oxybate has a more complex dosing schedule which includes dose titration (increasing the dose slowly) over several weeks. In addition, sodium oxybate is given twice at night, which may disrupt sleep.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Wakix is of significant benefit to patients affected by narcolepsy.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Wakix 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 Wakix 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.