EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

20 November 2015
EMA/CHMP/764032/2015
Press Office

Press release

Narcolepsy treatment recommended for approval

Wakix to target excessive daytime sleepiness and cataplexy

The European Medicines Agency (EMA) has recommended granting a marketing authorisation for Wakix (pitolisant) for the treatment of narcolepsy with or without cataplexy (sudden severe muscle weakness or loss of muscle control).

Narcolepsy is a rare, long-term sleep disorder which affects the brain's ability to regulate the normal sleep-wake cycle. This can lead to symptoms such as excessive daytime sleepiness including the sudden urge to sleep, and disturbed night-time sleep. Narcolepsy symptoms can be very severe and can have a significant impact on the private and professional life of patients. In addition, some patients may experience sudden episodes of a related condition, cataplexy, potentially causing dangerous falls and increasing the risks of accidents, including car accidents.

Wakix will add to the available treatment options for narcolepsy. It is a first-in-class medicine that acts on histamine H3 receptors in the brain. This leads to increased histamine release in the brain, thereby enhancing wakefulness and alertness.

EMA's Committee for Medicinal Products for Human Use (CHMP) evaluated all available data on the safety, efficacy and quality of Wakix. Its recommendation to authorise Wakix for narcolepsy patients with or without cataplexy was based on the examination of two pivotal placebo-controlled studies involving 259 patients, as well as one uncontrolled, open-label study in 102 patients with narcolepsy and one supportive study in 105 patients. The studies showed that Wakix was effective in reducing excessive daytime sleepiness in patients with narcolepsy. The beneficial effect of Wakix on cataplexy was shown in one of the pivotal studies as well as in the supportive study.

The studies did not identify major safety concerns with Wakix. Insomnia, headache and nausea were among the most common side effects observed in the clinical trials and the CHMP decided on measures to mitigate these risks.

In addition, the CHMP requested the company to carry out a long-term safety study in order to further investigate the safety of the medicine when patients use it over long periods of time.

Because narcolepsy is rare, Wakix received an orphan designation from the Committee for Orphan Medicinal Products (COMP) in 2007. Orphan designation is the key instrument available in the European Union (EU) to encourage the development of medicines for patients with rare diseases.

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Orphan-designated medicines qualify for ten years' market exclusivity. In addition orphan designation gives medicine developers access to incentives, such as fee reductions for marketing authorisation applications and scientific advice.

The opinion adopted by the CHMP at its November 2015 meeting is an intermediary step on Wakix's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, each Member State will take a decision on price and reimbursement based on the potential role/use of this medicine in the context of its national health system.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Wakix is Bioprojet Pharma.
3. Following this positive CHMP opinion, the Committee for Orphan Medicinal Products (COMP) will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu.

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