EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

24 April 2015
EMA/CHMP/260099/2015
Press Office

Press release

First EU treatment for rare sleep-wake disorder

Hetlioz to help regulate sleep patterns in blind people

The European Medicines Agency (EMA) has recommended granting a marketing authorisation for Hetlioz (tasimelteon) to treat non-24-hour sleep-wake disorder in totally blind adults. There is currently no approved treatment for this condition in the European Union (EU).

Non-24-hour sleep-wake disorder occurs almost exclusively in people who are completely blind. How the body adjusts to the 24-hour clock is closely linked to the pattern of daylight, and so people who do not perceive light are more likely to suffer from the disorder. They fall asleep and wake up at different times compared with the general population, often in a pattern that is closer to a 25-hour clock. As a result, they have problems adjusting to the standard timetable of everyday life, often being awake or asleep at unusual times.

Non-24-hour sleep-wake disorder is a long-term debilitating condition. The excessive daytime sleepiness affects quality of life and patients' ability to follow a normal daily schedule.

The hormone melatonin plays a key role in co-ordinating the body's sleep cycle by acting on receptors in specific areas of the brain. Melatonin is produced by the pineal gland in the brain during the hours of darkness. Hetlioz is a melatonin-receptor agonist. This means that it attaches to the receptors that melatonin normally attaches to. By attaching itself to these receptors, Hetlioz works by promoting sleep and regulating sleep patterns.

The effectiveness of Hetlioz was demonstrated in two clinical trials, which showed that treatment with Hetlioz resulted in significant improvement compared with placebo, both in increasing night-time sleep and decreasing daytime sleep duration.

The most common side effects reported with Hetlioz include headache, drowsiness, and nightmares or unusual dreams.

Because non-24-hour sleep-wake disorder is rare, Hetlioz received an orphan designation in 2011. Orphan designation and the associated incentives such as scientific advice are among the Agency's most important instruments to encourage the development of medicines for patients with rare diseases.

The opinion adopted by the Committee for Medicinal Products for Human Use (CHMP) at its April 2015 meeting is an intermediary step on Hetlioz's path to patient access. The CHMP opinion will now be sent

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

to the European Commission for the adoption of a decision on EU-wide marketing authorisation. Once marketing authorisation has been granted, a decision about price and reimbursement will then take place at the level of each Member State considering the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The marketing authorisation applicant for Hetlioz is Vanda Pharmaceuticals Limited, UK.
3. Following this positive CHMP opinion, the Committee for Orphan Medicinal Products (COMP) will assess whether the 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

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu