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Medicines licensing

First treatment for blind people with rare sleep disorder gets the go-ahead in Europe

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The European Medicines Agency has recommended approval for tasimelteon, a sleep regulation medication used to treat a rare sleep disorder in blind people.

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Non-24-hour sleep-wake disorder is most prevalent in blind people, which causes them to awaken or fall asleep at abnormal times

A drug to treat a rare sleep disorder almost exclusively affecting blind people has been recommended for authorisation by the European Medicines Agency (EMA).

The oral sleep regulation medication, tasimelteon (Hetlioz), is the first EU-approved treatment for the non-24-hour sleep-wake disorder. It was approved by the US Food and Drug Administration in January 2014.

The melatonin-receptor agonist treats a debilitating condition, in which patients have sleep patterns that are not adjusted to the standard 24-hour clock. Because the condition is an uncommon one, tasimelteon received an orphan designation – which promotes development of treatments for rare diseases – in 2011. The condition is estimated to affect between 76,000 and 112,000 blind people in the European Union, based on Eurostat data from the EU-27, Norway, Iceland and Liechtenstein.

Patients with non-24-hour sleep-wake disorder follow a pattern closer to a 25-hour clock, making it difficult for them to adjust to standard timetables and causing them to awaken or fall asleep at abnormal times. The disorder leads to excessive daytime sleepiness that can affect ability to follow a normal daily schedule. Since the body's adjustment to a 24-hour-clock is closely linked to daylight patterns, the disorder is most prevalent in people who are unable to perceive light.

As there is currently no approved treatment in the EU, patients are frequently prescribed medicines such as a benzodiazepine to help with sleep, or caffeine to help them stay awake.

The hormone melatonin, which is produced by the pineal gland in the brain during hours of darkness, is important for coordinating the sleep cycle by acting on receptors in specific areas of the brain. Tasimelteon attaches to melatonin receptors, helping to regulate sleep patterns.

The drug acts as a circadian regulator that resets the master body clock in the suprachiasmatic nucleus in the brain, according to US-based Vanda Pharmaceuticals, the company marketing the product. The suprachiasmatic nucleus contains about 20,000 nerve cells and is located in the hypothalamus, an area of the brain just above where the optic nerves from the eyes cross.

The drug's effectiveness has been demonstrated in two clinical trials, where it showed significant improvement in increasing night-time sleep and decreasing daytime sleep duration, when compared with a placebo, according to the EMA.

The most common side effects from the treatment included headache, drowsiness and nightmares.

The recommendation by the EMA's Committee for Medicinal Products for Human Use (CHMP) will now be forwarded to the European Commission, which will adopt a final decision on EU-wide marketing authorisation of tasimelteon.

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