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Wakix (*pitolisant*)

An overview of Wakix and why it is authorised in the EU

What is Wakix and what is it used for?

Wakix is used to treat narcolepsy in adults, adolescents and children from 6 years of age. Narcolepsy is a long-term sleep disorder which affects the brain's ability to regulate the normal sleep-wake cycle. This leads to symptoms such as an irresistible urge to sleep, even at inappropriate times and places, and disturbed night-time sleep. Some patients also have episodes of severe muscle weakness (cataplexy) that can cause collapse. Wakix is used in patients with or without cataplexy.

Narcolepsy is rare, and Wakix was designated an 'orphan medicine' (a medicine used in rare diseases) on 10 July 2007. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu307459.

Wakix contains the active substance pitolisant.

How is Wakix used?

Wakix can only be obtained with a prescription and treatment should be started by a doctor experienced in the treatment of sleep disorders.

Wakix is available as tablets to be taken by the mouth once a day in the morning during breakfast.

For more information about using Wakix, see the package leaflet or contact your doctor or pharmacist.

How does Wakix work?

The active substance in Wakix, pitolisant, blocks histamine from attaching to a receptor (target) on nerve cells called 'histamine H3 receptor'. As a result, more histamine is produced in the brain, which attaches to another type of receptor called 'histamine H1 receptor'. This increases the activity of certain brain cells called histamine neurons, which are important for regulating sleep and wakefulness.

What benefits of Wakix have been shown in studies?

Wakix has been investigated in 2 main studies involving a total of 261 adults with narcolepsy, the majority of whom also had cataplexy. The studies compared Wakix with placebo (a dummy treatment). The main measure of effectiveness was based on how sleepy patients felt during daytime, assessed

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using the Epworth Sleepiness Scale or ESS. This is a standard scale used in patients with narcolepsy which ranges from 0 to 24.

The first study showed that Wakix was more effective than placebo at reducing daytime sleepiness: patients taking Wakix had an average reduction of 3 points more in the ESS scale than those taking placebo after 8 weeks of treatment. Results from this study also showed a decrease in the number of cataplexy attacks. The second study, however, did not show a difference between Wakix and placebo at reducing sleepiness or cataplexy.

When looking at sleepiness with an objective test called Maintenance of Wakefulness Test (MWT), the results of the two studies together showed that Wakix significantly improved wakefulness compared with placebo.

In a third study involving 105 patients with narcolepsy and cataplexy, Wakix was also more effective than placebo at reducing the number of cataplexy attacks: the number of cataplexy attacks decreased from around 9 to 3 per week in patients taking Wakix, while it remained at around 7 per week in patients taking placebo.

A fourth study involved 110 children with narcolepsy from 6 to 17 years of age, the majority of whom also had cataplexy. In this study, the Ullanlinna Narcolepsy Scale (UNS), a questionnaire with scores ranging from 0 to 44, was used to measure sleepiness and the frequency and severity of cataplexy.

After 8 weeks of treatment, the average reduction in the UNS score was 4 points greater in children taking Wakix than in those taking placebo. In addition, the average reduction in the score for cataplexy was 2 points greater in patients taking Wakix than in those taking placebo.

What are the risks associated with Wakix?

The most common side effects with Wakix in adults (which may affect up to 1 in 10 people) are insomnia (difficulty sleeping), headache, nausea (feeling sick), anxiety, irritability, dizziness, depression, tremor, sleep disorders, tiredness, vomiting, vertigo (a spinning sensation) and dyspepsia (heartburn). Serious but rare side effects are abnormal loss of weight and spontaneous abortion. The side effects in children and adolescents are similar to those in adults. For the full list of side effects of Wakix, see the package leaflet.

Wakix must not be used in patients with severely reduced liver function and in women who are breastfeeding. For the full list of restrictions, see the package leaflet.

Why is Wakix authorised in the EU?

Studies show that Wakix is effective at reducing the two major symptoms of narcolepsy, excessive daytime sleepiness and cataplexy, in adults, adolescents and children from 6 years of age. In addition, Wakix works differently from currently available treatments and therefore offers an alternative treatment option for patients with narcolepsy. The safety profile of Wakix is considered acceptable, with no major safety concerns identified.

The European Medicines Agency therefore decided that Wakix’s benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Wakix?

The company that markets Wakix will carry out an observational study to collect information on the safety of the medicine when used in medical practice.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Wakix have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Wakix are continuously monitored. Suspected side effects reported with Wakix are carefully evaluated and any necessary action taken to protect patients.

Other information about Wakix

Wakix received a marketing authorisation valid throughout the EU on 31 March 2016.

Further information on Wakix can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/wakix.

This overview was last updated in 03-2023.