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Ztalmy (*ganaxolone*)

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

What is Ztalmy and what is it used for?

Ztalmy is a medicine used to treat epileptic seizures in children from 2 to 17 years of age who have a condition known as cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder. These patients can continue taking Ztalmy when they become adults if a clear benefit has been observed.

The medicine is used in combination with other anti-epileptic medicines.

CDKL5 deficiency disorder is rare, and Ztalmy was designated an 'orphan medicine' (a medicine used in rare diseases) on 13 November 2019. Further information on the orphan designation can be found on the EMA website.

Ztalmy contains the active substance ganaxolone.

How is Ztalmy used?

The medicine can only be obtained with a prescription, and treatment should be started and supervised by a doctor who has experience in treating patients with epilepsy.

Ztalmy is available as a liquid to be taken by mouth and is usually given three times a day. The dose is determined by the patient's weight.

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

How does Ztalmy work?

The active substance in Ztalmy, ganaxolone, mimics the action of a substance in the body called allopregnanolone. It switches on so-called GABA receptors, which reduces excessive electrical activity in the brain and thus lowers the number of seizures.

What benefits of Ztalmy have been shown in studies?

A main study showed that Ztalmy reduces the frequency of seizures in children and adolescents with CDKL5 deficiency disorder who are taking at least one other epilepsy medicine.

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The study involved a total of 101 patients with CDKL5 deficiency disorder and compared Ztalmy with placebo (a dummy treatment), both given in addition to existing epilepsy medicines.

On average, the monthly number of major seizures was reduced by 29% in the group of patients treated with Ztalmy, and by 6% in the group treated with placebo.

What are the risks associated with Ztalmy?

For the full list of side effects and restrictions with Ztalmy, see the package leaflet.

The most common side effects with Ztalmy (which may affect more than 1 in 10 people) include sleepiness and fever.

Why is Ztalmy authorised in the EU?

The main study showed that Ztalmy is effective at reducing the number of seizures in children with CDKL5 deficiency disorder. The side effects are considered manageable. The European Medicines Agency therefore decided that Ztalmy'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 Ztalmy?

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

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

Other information about Ztalmy

Ztalmy received a marketing authorisation valid throughout the EU on 26 July 2023.

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

This overview was last updated in 06-2023.