25 May 2023
EMA/CHMP/207481/2023
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Ztalmy

ganaxolone

On 25 May 2023, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Ztalmy[2], intended for the treatment of epileptic seizures associated with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD) in children and adolescents. The applicant for this medicinal product is Marinus Pharmaceuticals Emerald Limited.

Ztalmy will be available as a 50 mg/ml oral suspension. The active substance of Ztalmy is ganaxolone, an antiepileptic (ATC code: N03AX27). The precise mechanism of action is not known but its anticonvulsant effects are thought to result from a positive allosteric modulation of the $GABA_A$ receptors.

The benefits of Ztalmy lie in its ability to reduce the frequency of the seizures associated with CDD as demonstrated in a double-blind, randomised, placebo-controlled study in children and adolescents. The most common side effects are somnolence and pyrexia.

The full indication is:

> Ztalmy is indicated for the adjunctive treatment of epileptic seizures associated with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD) in patients 2 to 17 years of age. Ztalmy may be continued in patients 18 years of age and older.

The treatment should be initiated and supervised by physicians with experience in the treatment of epilepsy.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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