Drugs

PEER-REVIEWED FEATURE

Ganaxolone: Adis Evaluation

Key Points

- A positive allosteric $GABA_A$ receptor modulator is being developed by Marinus Pharmaceuticals for the treatment of epileptic disorders
- Received its first approval on 18 March 2022 in the USA
- Approved for use in the treatment of seizures associated with CDD in patients ≥ 2 years of age

Summary

Ganaxolone (ZTALMY®; Marinus Pharmaceuticals) is a synthetic neuroactive steroid that acts as a positive allosteric modulator of the gamma-aminobutyric acid $(GABA)_A$ receptor complex. Ganaxolone received its first approval in March 2022 in the USA for the treatment of seizures associated with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD) in patients 2 years of age and older. Approval was based on the results of a multinational phase III trial, in which ganaxolone was effective in reducing seizure frequency in children and adolescents with CDD. In the EU, a Marketing Authorization Application has been filed for ganaxolone in the treatment of seizures associated with CDD and an opinion from the Committee for Medicinal Products for Human Use is expected later this year. Oral ganaxolone is also currently undergoing phase III evaluation in the treatment of tuberous sclerosis complex-related epilepsy, while an intravenous formulation of ganaxolone is being evaluated in refractory status epilepticus. This article summarizes the milestones in the development of ganaxolone leading to this first approval for seizures associated with CDD.

Lamb, Y. N. Drugs. 2022. https://doi.org/10.1007/s40265-022-01724-0