Long-term treatment with ganaxolone for seizures in CDKL5 Deficiency Disorder

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The purpose of this summary is to help you understand the findings from a recent article related to ganaxolone.

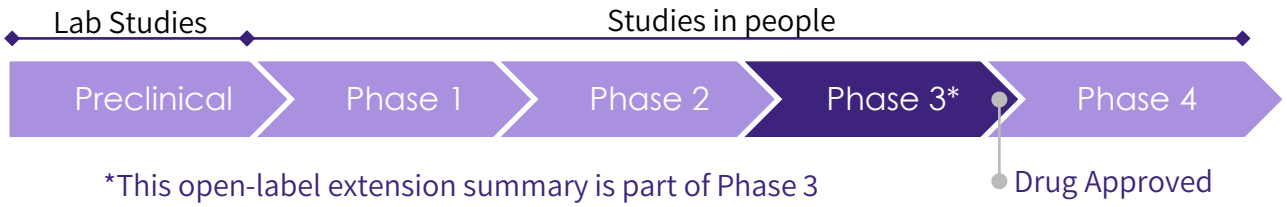
- Ganaxolone is approved in the United States for the treatment of seizures associated with CDKL5 Deficiency Disorder in patients 2 years of age and older.
- Researchers look at the results of clinical studies to understand if a drug works, how it works, and most importantly, if it is safe to give to patients.
- Clinical trials to get a drug approved include Phase 3 studies which compare the drug being studied to a placebo. Once patients complete this phase, they may have the option to enter an open-label extension (OLE) phase where everyone is on treatment and followed for safety and other outcomes.
- This summary shares the findings from a recent open-label study of a new treatment.

Study Information

Patients with CDKL5 Deficiency Disorder have a rare and severe genetic disorder that causes seizures plus delays in learning and muscle coordination. Ganaxolone works in the brain by making it less over-active which can help decrease seizures. This long-term open-label extension only included patients who completed the first phase of the study where they received ganaxolone or a placebo. In this study, every patient received ganaxolone and took it for up to two years. This summary will explain what was learned from the study.

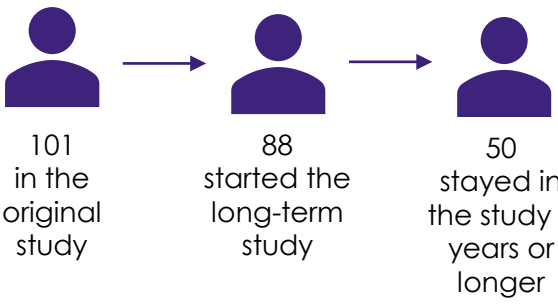
How do you say **Ganaxolone?** (gan AX oh lone)

Time in Drug Development



*This open-label extension summary is part of Phase 3

Study Patients



- All patients had CDKL5 Deficiency Disorder
- Patients were between 2 and 19 years old
- 8 out of 10 patients were female which reflected the normal distribution in CDKL5
- At baseline (before entering the phase 3 study), patients were taking 2 anti-seizure medications and had around 50 seizures per month
- 4 out of 10 patients were from the United States

Results of the Study

- After two years, the 50 patients still being treated had about half as many seizures per month than their baseline (48% fewer seizures)
- For these same 50 patients, nearly half (46%) had more than half of their seizures stop and about a quarter (24%) of patients had a drop of more than 3/4 of the number of seizures that they had at baseline
- Patients also had 4 more days per month without any seizures
- Over two years, 37 patients stopped taking the drug mostly due to it not working well enough, side effects, or they decided to stop being in the study
- The side effects that patients reported the most were sleepiness, other seizures, and not being hungry

Seizures

↓48%

per month compared to baseline seizure frequency

At two years,

3 out of 4

baseline seizures per month were no longer happening for 24% of patients

Main Conclusions

- After being treated with ganaxolone for two years, most patients continued to have fewer seizures and fewer days with seizures, supporting the idea that ganaxolone continues to be effective over a longer period time
- There were no new safety concerns with the drug

Funding for this study was provided by the study sponsor, Marinus Pharmaceuticals, Inc.

Marinus would like to thank everyone who took part in this study – most importantly the patients and families who committed significant time and effort to help others.

The full title of this article is: *Long-term treatment with ganaxolone for seizures associated with cyclin-dependent kinase-like 5 deficiency disorder: 2-year open-label extension follow-up*

You can access the full article here: https://pubmed.ncbi.nlm.nih.gov/37950390/

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