MEDICAL MUTUAL®

Drug Policy

Policy:	Ztalmy (ganaxolone)	Annual Review Date: 08/24/2023 Last Revised Date: 08/24/2023

OVERVIEW

Ztalmy, a neuroactive steroid gamma-aminobutyric acid (GABA) A receptor positive modulator, is indicated for the treatment of seizures associated with cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder (CDD) in patients ≥ 2 years of age.

POLICY STATEMENT

This policy involves the use of Ztalmy. Prior authorization is recommended for pharmacy benefit coverage of Ztalmy. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Ztalmy as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Ztalmy be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Ztalmy is recommended in those who meet the following criteria:

1. **Seizures associated with Cyclin-Dependent Kinase-Like 5 (CDKL5) Deficiency Disorder**
   Criteria. *Patient must meet the following criteria*
   - **A.** Patient is ≥ 2 years of age; AND
   - **B.** Patient has molecularly confirmed pathogenic or likely pathogenic mutation in the CDKL5 gene; AND
   - **C.** The medication is prescribed by or in consultation with a neurologist; AND
   - **D.** The patient has tried at least two antiseizure medications, each from a different pharmacologic class, unless an intolerance or contraindication exists [documentation required];
     Note: Examples of AEDs include valproate, levetiracetam, clobazam and vigabatrin

Initial Approval/ Extended Approval.

A) *Initial Approval:* 6 months (180 days)
B) *Extended Approval:* 1 year (365 days)

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Ztalmy has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Ztalmy® oral suspension [prescribing information]. Radnor, PA: Marinus; March 2022.
2. Olson HE, Daniels CI, Haviland I, et al. Current neurologic treatment and emerging therapies in CDKL5 deficiency disorder. *J Neurodev Disord*. 2021;13(1):40.
3. International Foundation for CDKL5 Research. About CDKL5. Available at: https://www.cdkl5.com/about-cdkl5/. Accessed on July 5, 2022.
4. Knight EMP, Amin S, Bahi-Buisson N, et al. Safety and efficacy of ganaxolone in patients with CDKL5 deficiency disorder: results from the double-blind phase of a randomised, placebo-controlled, phase 3 trial. *Lancet Neurol*. 2022;21:417-427.