Vykat XR (diazoxide choline)

Disclaimer

Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.

Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.

Summary

Prader-Willi syndrome (PWS) is a genetic multisystem disorder resulting from the loss of function of specific genes on chromosome 15, leading to disruption in the regular function of the hypothalamus. Hyperphagia associated with PWS is present in about 90-100% of individuals and is hypothesized to be caused by a hypothalamic abnormality that causes a lack of satiety. The onset of weight gain and increased appetite begins around 3 years of age and progresses to extreme hyperphagia between 5 and 13 years of age. Upon reaching adulthood, some individuals hyperphagia may lessen over time and satiety can occur.

The exact mechanism of action of diazoxide choline in the treatment of hyperphagia with PWS is unknown.

Definitions

"Body fat mass" is the total amount of lipids in the body.

"Documentation" refers to written information, including but not limited to:

- Up-to-date chart notes, relevant test results, and/or relevant imaging reports to support diagnoses; or
- Prescription claims records, and/or prescription receipts to support prior trials of formulary alternatives.

"Hyperphagia" is abnormally increased or excessive hunger.

"Leptin" is made by fat cells and it regulates energy balance in the brain. In PWS, a prolonged increase in leptin causes leptin resistance and subsequently lack of satiety, overeating, and increased weight.

"No evidence of" indicates that the reviewer has not identified any records of the specified item or condition within the submitted materials or claims history. In the absence of such evidence, the member is considered eligible. If any evidence of the item or condition is present upon review of the request, the member does not qualify.

"[s]" indicates state mandates may apply.

"Satiety" is the feeling of fullness and satisfaction after eating.

Clinical Indications

Medical Necessity Criteria for Clinical Review

General Medical Necessity Criteria

The Plan considers Vykat XR medically necessary when ALL of the following criteria are met:

1. The medication is prescribed by or in consultation with an endocrinologist or psychiatrist; *AND*
2. The member is 4 years of age or older; *AND*
3. The member weighs 20 kg or greater; *AND*
4. The member's weight (within the last 45 days) is provided; *AND*
5. Vykat XR is being prescribed at a dose and frequency that is within FDA approved labeling (see Appendix A, Table 1); *AND*

   The requested medication is being used within the Plan's Quantity Limit of:

   - *25 mg: 4 tablets per day*
   - *75 mg: 3 tablets per day*
   - *150 mg: 3 tablets per day*
6. The member meets the applicable Medical Necessity Criteria for Initial Clinical Review or Subsequent Clinical Review listed below.

Medical Necessity Criteria for Initial Clinical Review

Initial Indication-Specific Criteria

Prader-Willi syndrome (PWS)

The Plan considers Vykat XR medically necessary when ALL of the following criteria are met:

7. The member meets the above General Medical Necessity Criteria; *AND*
8. The member has a diagnosis of Prader-Willi syndrome (PWS); *AND*
9. The diagnosis is supported by genetic testing documentation demonstrating ONE (1) of the following:
   a. Deletion of the chromosomal 15q11-Q13 region; *or*
   b. Maternal uniparental disomy in chromosome 15; *or*
   c. Imprinting defects, translocations, or inversions involving chromosome 15 that have been described to cause PWS; *AND*
10. The member has moderate to severe hyperphagia supported by TWO (2) of the following characteristics:
    a. Aggressive food seeking behavior (e.g., nighttime food seeking, persistent requests for food); *or*
    b. Bargaining or manipulation to obtain more food; *or*
    c. Distress when denied access to food; *or*
    d. Food obsession or preoccupation that interferes with normal daily activities (e.g., self-care, recreation, school or work); *or*
    e. Foraging through trash for food; *or*

f. Hoarding, sneaking, or stealing food; *AND*

11. The member has been assessed for hyperglycemia, and if the member has hyperglycemia the member's blood glucose will be optimized prior to initiating treatment.
12. The member meets ALL of the following:
    a. No evidence of renal impairment; *and*
    b. No evidence of hepatic impairment; *and*
    c. No evidence of hyperinsulinemic hypoglycemia; *and*
    d. No evidence of known hypersensitivity to diazoxide or thiazides.

If the above prior authorization criteria are met, the requested product will be authorized for up to 6-months.[s]

Continued Care

Medical Necessity Criteria for Subsequent Clinical Review

Subsequent Indication-Specific Criteria

Prader-Willi syndrome (PWS)

The Plan considers Vykat XR medically necessary when ALL of the following criteria are met:

1. The member meets the above applicable General Medical Necessity Criteria; *AND*
2. The member has experienced a documented improvement in hyperphagia with PWS. This improvement must be validated by clinical documentation (within the last 6 months) showing a reduction in ONE (1) of the following:
    a. Hyperphagia; *or*
    b. Body fat mass; *or*
    c. Levels of leptin; *AND*
3. There is no evidence of unacceptable toxicity or adverse reactions to Vykat XR.

If the above reauthorization criteria are met, the requested product will be authorized for up to 12-months.[s]

Experimental or Investigational or unproven[s]

Vykat XR for any other indication or use is considered experimental, investigational, or unproven. Non-covered indications include, but are not limited to, the following:

- Mild hyperphagia as clinical trials did not include this subpopulation; therefore, the safety and effectiveness of Vykat XR have not been established in those with mild hyperphagia.
- Hypoglycemia due to hyperinsulinism.
- Inoperable islet cell adenoma or carcinoma, or extrapancreatic malignancy.
- Leucine sensitivity, islet cell hyperplasia, nesidioblastosis, extrapancreatic malignancy, islet cell adenoma, or adenomatosis.

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Appendix A

Table 1: Recommended Starting Dosage and Titration Regimen in Adults and Pediatric Patients 4 Years of Age and Older

Weight	Recommended Once Daily Dosage			
	Starting Dosage	Titration Dosage	Titration Dosage	Target Maintenance Dose
	Weeks 1 and 2	Weeks 3 and 4	Weeks 5 and 6	
20 kg to <30 kg	25 mg	50 mg	75 mg	100 mg
30 kg to <40 kg	75 mg	150 mg	150 mg	150 mg
40 kg to <65 kg	75 mg	150 mg	225 mg	225 mg
65 kg to <100 kg	150 mg	225 mg	300 mg	375 mg
100 kg to <135 kg	150 mg	300 mg	375 mg	450 mg
≥135 kg	150 mg	300 mg	450 mg	525 mg

The maximum recommended dosage of Vykat XR is 5.8 mg/kg/day or 525 mg per day. Dosages above 5.8 mg/kg/day or 525 mg per day have not been evaluated in individuals with PWS.

Clinical Guideline Revision / History Information

Original Date: 01/01/2026
Reviewed/Revised: 10/01/2026