Pharmacy Policy Bulletin: J-1451 Vykat XR (diazoxide choline) – Commercial and Healthcare Reform	
Number: J-1451	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.):** **1.** Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-1451-001	**Original Date:** 06/25/2025
Effective Date: 07/18/2025	**Review Date:** 06/25/2025

Drugs Product(s):	• Vykat XR (diazoxide choline)
FDA-Approved Indication(s):	• Treatment of hyperphagia in adults and pediatric patients 4 years of age and older with Prader-Willi syndrome (PWS).

Background:	• Vykat XR is the first FDA-approved drug for PWS; it targets its hallmark symptom, hyperphagia. • PWS is a genetic, multisystem disorder condition that leads to physical, mental, and behavioral problems. The hallmark symptom of PWS is hyperphagia. Other characteristics of PWS include behavioral problems, cognitive disabilities, low muscle tone, short stature, excess body fat accumulation, developmental delays, and incomplete sexual development. PWS results from an abnormality in gene expression on chromosome 15; a definitive diagnosis is based on genetic testing of individuals that exhibit typical clinical features. Males, females, all races, and ethnicities can be equally affected by this condition. PWS affects between 350,000 and 400,000 individuals worldwide. • During infancy, most children with PWS have feeding and swallowing difficulties leading to poor weight gain. This is followed by hyperphagia in early childhood leading to an increased appetite or food consumption, reduced satiety, as well as disruptive food-related behaviors, including aggressive food seeking. The hyperphagia continues throughout life. Hyperphagia can lead to significant mortality due to stomach rupture and choking, as well as longer-term co-morbidities such as diabetes, obesity, and cardiovascular disease. PWS is considered the most common cause of genetically determined obesity in children. • Although the exact mechanism of action of Vykat XR in the treatment of hyperphagia with PWS is unknown, it is thought to work by stimulating ion flux through the adenosine triphosphate (ATP)-sensitive potassium channels in the hypothalamus.

- The recommended starting dosage and titration schedule is based on the patient's body weight:

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

- Prescribing Considerations:
  - Prior to initiation of therapy, obtain a fasting plasma glucose and HbA1c; optimize blood glucose in patients with hyperglycemia.
  - During treatment, continue to monitor fasting glucose and HbA1c.
  - Recommended starting dosage, titration schedule, and target maintenance dose is based on patient's body weight. Dose is administered orally, once daily.
  - Monitor patient for signs and symptoms of edema or fluid overload.
  - Do not substitute diazoxide oral suspension for Vykat XR (diazoxide choline).
  - Use is contraindicated in patients with hypersensitivity to thiazides.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Vykat XR may be approved when all of the following criteria are met **(A., B., and C.)**:

A. The member is 4 years of age or older.

B. The member has a diagnosis of Prader-Willi syndrome (ICD-10: Q87.11) confirmed by documentation of genetic analysis with identification of abnormal DNA methylation of chromosome 15q11.2-q13.

C. The member is experiencing hyperphagia due to the PWS diagnosis.

II. Reauthorization

When a benefit, reauthorization of Vykat XR may be approved when the following criterion is met **(A.)**:

A. The prescriber attests that the member has experienced a decrease in hyperphagic and food-related behaviors.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.

II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Vykat XR. [package insert]. Redwood City, CA: Soleno Therapeutics, Inc.; March 2025.
2. Rare Disease Advisor. Prader-Willi Syndrome (PWS). Available at: https://www.rarediseaseadvisor.com/disease-info-pages/prader-willi-syndrome-overview/. Accessed April 10, 2025.
3. UpToDate. Prader-Willi Syndrome: Clinical Features and Diagnosis. Available at: https://www.uptodate.com. Accessed April 10, 2025.
4. Butler MG, Miller JL, Forster JL. Prader-Willi Syndrome – Clinical Genetics, Diagnosis and Treatment Approaches: An Update. *Curr Pediatr Rev.* 2019;15(4): 207-244.
5. Drugs.com. Vykat XR FDA Approval History. Available at: https://www.drugs.com/history/vykat-xr.html. Accessed April 10, 2025.
6. Mahmoud R, Kimonis V, Butler MG. Clinical Trials in Prader-Willi Syndrome: A Review. *Int. K. Mol. Sci.* 2023, 24, 2150.
7. Soleno Therapeutics. About Prader-Willi Syndrome. Available at: https://www.soleno.life/for-patients-and-families/. Accessed April 11, 2025.
8. Soleno Therapeutics. Soleno Therapeutics Announces U.S. FDA Approval of Vykat XR to Treat Hyperphagia in Prader-Willi Syndrome. Available at: https://www.investors.soleno.life/news-releases/news-release-details/soleno-therapeutics-announces-us-fda-approval-vykattm-xr-treat. Accessed March 31, 2025.