VENTURA COUNTY
HEALTH CARE PLAN

PRIOR AUTHORIZATION POLICY

POLICY: Prader-Willi Syndrome – Vykat XR Prior Authorization Policy
- Vykat™ XR (diazoxide choline extended-release tablets – Soleno)

REVIEW DATE: 04/02/2025

OVERVIEW

Vykat XR, a potent activator of the adenosine triphosphate-sensitive potassium channel[2], is indicated for the treatment of hyperphagia in adults and pediatric patients 4 years of age and older with Prader-Willi syndrome.[1]

Diazoxide increases blood glucose, primarily through inhibiting insulin release from the pancreas. The exact mechanism of action of diazoxide choline in the treatment of hyperphagia in patients with Prader-Willi syndrome is unknown.[1]

Disease Overview

Prader-Willi syndrome is a rare, genetic combination neurobehavior and metabolic disorder.[2] It has an estimated birth incidence of 1:15,000 to 1:20,000. The condition is caused from lack of expression of paternally inherited imprinted genes on chromosome 15q11.2-q13. The diagnosis is established by identification of 'abnormal' DNA methylation within the Prader-Willi critical region at 15q11.2-q13.[3] Cases generally occur sporadically. Patients with Prader-Willi syndrome are characterized in infancy hypotonia with poor appetite. As the child ages, clinical characteristics include developmental delay, mild cognitive impairment, hypogonadism, and hyperphagia (with central obesity). It has been determined that 90 to 100% of patients with Prader-Willi syndrome will have hyperphagia and obesity.[3]

Clinical Efficacy

The efficacy of Vykat XR was established in one 16-week, double-blind, placebo-controlled, randomized withdrawal study.[1] Prior to the pivotal study, patients were on Vykat XR for a mean duration of approximately 3 years. During the withdrawal of Vykat XR, 77 patients were randomized 1:1 to either continue their current dose of Vykat XR or use placebo. The primary endpoint was the change in baseline in the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) total score. The HQ-CT total score ranges from 0 to 36 (higher scores indicate greater severity of hyperphagia and food-related behaviors). At baseline, the mean score was 9.0 in the Vykat XR group and 8.1 in the placebo group. At Week 16, the least square (LS) mean change from baseline was 2.6 for the Vykat XR group and 7.5 for the placebo group. There was a statistically significant 'worsening' in the placebo group with a LS mean difference of -5.0 (95% confidence interval: -8.1, -1.8). The placebo group also experienced significantly greater increases in weight, body mass index (BMI), and BMI z-scores.[5]

Guidelines

Vykat XR is not addressed in clinical guidelines. European guidelines on Prader-Willi syndrome (2024) state that hyperphagia and food-seeking behaviors begin in early childhood and persist throughout the patient's life.[4] Food security practices can include physical interventions, such as locks on refrigerators or cupboards. Guidelines additionally mention dietary strategies and exercise programs to prevent weight gain. There are no consensus guidelines on the use of anti-obesity medications (e.g., topiramate, metformin, liraglutide) in Prader-Willi syndrome.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Vykat XR. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Vykat XR as well as the monitoring required for adverse events and long-term efficacy, approval requires Vykat XR to be prescribed by or in consultation with a physician who specializes in the condition being treated.

RECOMMENDED AUTHORIZATION CRITERIA

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

FDA-Approved Indication

1. **Prader-Willi Syndrome.** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
   - **A)** Patient is ≥ 4 years of age; AND
   - **B)** The diagnosis of Prader-Willi syndrome has been established by identification of abnormal DNA methylation of chromosome 15q11.2Q13; AND
   - **C)** Patient has hyperphagia; AND
   - **D)** The medication has been prescribed by or in consultation with an endocrinologist.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Vykat XR is not recommended in the following situations:

1. **Hyperphagia in a patient without Prader-Willi syndrome.** Vykat XR tablets are only indicated for the treatment of hyperphagia in patients with Prader-Willi syndrome.[1] No data is available on the treatment of hyperphagia in patients without Prader-Willi.

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

REFERENCES

1. Vykat™ XR (diazoxide choline) extended-release tablets [prescribing information]. Redwood City, CA: Soleno; March 2025.
2. Miller JL, Gevers E, Bridges N, et al. Diazoxide choline extended-release tablet in people with Prader-Willi Syndrome: A double-blind, placebo-controlled trial. *J Clin Endocrinol Metab.* 2023;108(7):1676-1685.
3. Driscoll DJ, Miller JL, Cassidy SB. Prader-Willi Syndrome. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReview® [Internet]. Updated December 5, 2024. Available at: www.ncbi.nlm.nih.gov/books/NBK1330/pdf/Bookshelf_NBK1330.pdf. Accessed on March 20, 2025.
4. Shaikh MG, Barrett TB, Bridges N, et al. Prader-Willi syndrome: guidance for children and transition into adulthood. *Endocr Connect.* 2024; 13(8):e240091.
5. Gevers EF, Miller JL, Bridges NA, et al. Withdrawal of DCCR (diazoxide choline) extended-release tablets worsens hyperphagia and increases weight and BMI in a 16-week double-blind, placebo-controlled, randomized withdrawal period in patients with Prader-Willi syndrome. *J Endocr Soc.* 2024;8(Suppl 1):bvae163.055.

HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	04/02/2025