Clinical Policy: Diazoxide Choline (Vykat XR)

Reference Number: PA.CP.PHAR.701
Effective Date: 05/2025
Last Review Date: 04/2025

Description

Diazoxide choline (Vykat™ XR) is an adenosine triphosphate (ATP)-dependent potassium channel agonist.

FDA Approved Indication(s)

Vykat XR is indicated for the treatment of hyperphagia in adults and pediatric patients 4 years of age and older with Prader-Willi syndrome (PWS).

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Vykat XR is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Prader-Willi Syndrome (must meet all)**:**

1. Diagnosis of PWS confirmed by genetic testing;
2. Prescribed by or in consultation with anendocrinologist or geneticist;
3. Age ≥ 4 years;
4. Weight ≥ 20 kg;
5. Member has hyperphagia associated with PWS;
6. Dose does not exceed both of the following (a and b):
   a. 5.8 mg/kg per day;
   b. 525 mg per day.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Prader-Willi Syndrome (must meet all)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy as evidenced improvement in hyperphagia or other manifestations related PWS;
3. If request is for a dose increase, new dose does not exceed both of the following (a and b):
   a. 5.8 mg/kg per day;

b. 525 mg per day.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ATP: adenosine triphosphate
FDA: Food and Drug Administration
HQ-CT: hyperphagia questionnaire for clinical trials
PWS: Prader-Willi syndrome

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): known hypersensitivity to diazoxide, other components of Vykat XR, or to thiazides
- Boxed warning(s): none reported

Appendix D: Hyperphagia Questionnaire for Clinical Trials (HQ-CT)

The HQ-CT is a 9-question, caregiver completed questionnaire intended to assess a range of hyperphagia-related behaviors in PWS over a 2-week recall period. Responses range from 0 to 4 units each, with a total possible score of 0-36, and higher scores reflect more extreme hyperphagia.

HQ-CT Item	
1.	Upset when denied food
2.	Try to bargain or manipulate
3.	Forage through trash for food
4.	Get up at night to food seek
5.	Persistence after being told no more food
6.	Time spent talking about food
7.	Try to sneak or steal food
8.	Distress when told to stop food-related talk
9.	Interference with daily activities from food-related talk or behavior
Total (max score = 36)	

V. Dosage and Administration

<table>
<tr><th>Indication</th><th>Dosing Regimen</th><th>Maximum Dose</th></tr>
<tr><td>PWS</td><td>Vykat XR is administered orally once daily with the starting dosage and titration schedule based on body weight. The maximum recommended dosage is 5.8 mg/kg/day or 525 mg per day.
<table>
<tr><th rowspan="2">Weight</th><th>Starting Dosage</th><th>Titration Dosage</th><th>Titration Dosage</th><th rowspan="2">Target Maintenance Dosage</th></tr>
<tr><th>Weeks 1 and 2</th><th>Weeks 3 and 4</th><th>Weeks 5 and 6</th></tr>
<tr><td>20 to < 30 kg</td><td>25 mg</td><td>50 mg</td><td>75 mg</td><td>100 mg</td></tr>
<tr><td>30 to < 40 kg</td><td>75 mg</td><td>150 mg</td><td>150 mg</td><td>150 mg</td></tr>
<tr><td>40 to < 65 kg</td><td>75 mg</td><td>150 mg</td><td>225 mg</td><td>225 mg</td></tr>
<tr><td>65 to < 100 kg</td><td>150 mg</td><td>225 mg</td><td>300 mg</td><td>375 mg</td></tr>
<tr><td>100 to < 135 kg</td><td>150 mg</td><td>300 mg</td><td>375 mg</td><td>450 mg</td></tr>
<tr><td>≥ 135 kg</td><td>150 mg</td><td>300 mg</td><td>450 mg</td><td>525 mg</td></tr>
</table></td><td>5.8 mg/kg/day or 525 mg/day</td></tr>
</table>

VI. Product Availability

Extended-release tablets: 25 mg, 75 mg, 150 mg

VII. References

1. Vykat XR Prescribing Information. Redwood City, CA: Soleno Therapeutics, Inc.; March 2025. Available at: https://www.vykatxrhcp.com/prescribing-information.pdf. Accessed April 4, 2025.
2. ClinicalTrials.gov. A study of diazoxide choline in patients with Prader-Willi syndrome. Available at: https://clinicaltrials.gov/study/NCT03440814. Accessed April 15, 2024.
3. ClinicalTrials.gov. Open-label extension study of DCCR in PWS followed by double-blind, placebo-controlled, randomized withdrawal period. Available at: https://clinicaltrials.gov/study/NCT03714373. Accessed April 15, 2024.
4. 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. The Journal of Clinical Endocrinology & Metabolism 2023. 108(7):1676-1685.
5. Miller JL, Gevers E, Bridges N, et al. Diazoxide choline extended-release tablet in people with Prader-Willi syndrome: results form long-term open-label study. Obesity (Silver Spring). 2024;32(2):252-261.
6. Schwartz L, Ciaxas A, Dimitropoulos A, et al. Behavioral features in Prader-Willi syndrome (PWS): consensus paper from the International PWS clinical trial consortium. Journal of Neurodevelopmental Disorders. 2021;13(25):1-13.

Reviews, Revisions, and Approvals	Date
Policy created	04/2025