Clinical Policy: Diazoxide Choline (Vykat XR)

Reference Number: CP.PHAR.701
Effective Date: 03.26.25
Last Review Date: 05.25
Line of Business: Commercial, HIM, Medicaid Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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 health plans affiliated with Centene Corporation® 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 a pediatric endocrinologist or geneticist;
3. Age ≥ 4 years;
4. Weight ≥ 20 kg;
5. Documentation of baseline hyperphagia questionnaire for clinical trials (HQ-CT) score ≥ 13 (*see Appendix D)*;
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 (must meet 1 or 2)**:**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or

b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

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

1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B)*;
2. Member is responding positively to therapy as evidenced by reduction in overall HQ-CT total score from baseline;
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. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

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

Indication	Dosing Regimen	Maximum Dose
PWS	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.	5.8 mg/kg/day or 525 mg/day

Indication	Dosing Regimen					Maximum Dose
	Weight	Starting Dosage Weeks 1 and 2	Titration Dosage Weeks 3 and 4	Titration Dosage Weeks 5 and 6	Target Maintenance Dosage	
	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	

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	P&T Approval Date
Policy created pre-emptively	10.22.24	11.24
Drug is now FDA approved – criteria updated per FDA labeling: removed upper weight limit of ≤ 135 kg; removed the following	04.04.25	05.25

Reviews, Revisions, and Approvals	Date	P&T Approval Date
parameters for continued therapy: CGI-I, GI-C, body composition components, and behavioral improvements; for continued therapy, from revised continued approval duration from 6 months to 12 months; references reviewed and updated.		

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.