Vykat XR™ (diazoxide choline) – New orphan drug approval

- On March 26, 2025, Soleno Therapeutics announced the FDA approval of Vykat XR (diazoxide choline), for the **treatment of hyperphagia in adults and pediatric patients 4 years of age and older with Prader-Willi syndrome (PWS)**.

- Hyperphagia is the key symptom of PWS, a rare genetic neurodevelopmental disorder affecting 1 per 15,000 live births. Patients with hyperphagia chronically experience extreme hunger with inappropriate insatiability, leading to intense food-related behaviors, reduced quality of life, and increased mortality.

- Vykat XR is the first FDA approved treatment for hyperphagia in PWS.

    — Diazoxide is available as an oral suspension for the management of hypoglycemia due to hyperinsulinism. The suspension formulation and Vykat XR are not interchangeable.

- The exact mechanism of Vykat XR in the treatment of hyperphagia in PWS is unknown.

- The efficacy of Vykat XR was established in a 16-week, double-blind, placebo-controlled, randomized withdrawal study that followed an open-label study of Vykat XR in 77 adults and pediatric patients ages 4 years and older with PWS. The primary endpoint was the change from baseline at week 16 in the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) score, an observer-reported measure of hyperphagic and food-related behaviors where higher scores represent greater disease severity.

    — At week 16, there was statistically significant worsening of hyperphagia with placebo relative to Vykat XR. The least square mean change in HQ-CT scores were 2.6 and 7.6 for Vykat XR and placebo, respectively (difference -5.0, 95% CI: -8.1, -1.8).

- Vykat XR is contraindicated in patients with hypersensitivity to diazoxide, other components of Vykat XR, or thiazides.

- Warnings and precautions for Vykat XR include hyperglycemia (including diabetic ketoacidosis) and edema (including severe reactions of fluid overload).

- The most common adverse reactions (≥ 10% and at least 2% greater than in placebo) with Vykat XR use were hypertrichosis, edema, hyperglycemia, and rash.

- The recommended oral daily dose of Vykat XR is based on a weight-based titration over 6 weeks to target maintenance doses of:

    — 100 mg for patients 20 to < 30 kg
    — 150 mg for patients 30 to < 40 kg
    — 225 mg for patients 40 to < 65 kg
    — 375 mg for patients 65 to < 100 kg
    — 450 mg for patients 100 to < 135 kg
    — 525 mg for patients ≥ 135 kg.

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- Soleno Therapeutics plans to launch Vykat XR starting in April 2025. Vykat XR will be available as 25 mg, 75 mg, and 150 mg extended-release tablets.

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