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Soleno Therapeutics Publishes Pivotal VYKAT XR Study Results in Leading Endocrinology Journal

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KEY INSIGHTS

- Soleno Therapeutics announced publication of pivotal randomized withdrawal study results for VYKAT XR in the Journal of Clinical Endocrinology and Metabolism, demonstrating significant worsening of hyperphagia when treatment was withdrawn compared to continued administration.
- The 16-week study involving 77 participants showed statistically significant increases in hyperphagia scores with placebo versus VYKAT XR (P=0.0022), along with greater weight gain in the placebo cohort.
- VYKAT XR represents the first and only FDA-approved treatment for hyperphagia in Prader-Willi syndrome, addressing a critical unmet medical need for this rare genetic disorder affecting one in 15,000 live births.

Soleno Therapeutics has announced the publication of pivotal study results for **VYKAT XR** (**diazoxide choline** extended-release tablets) in the peer-reviewed Journal of Clinical Endocrinology and Metabolism, providing additional controlled data supporting the efficacy and safety of the first FDA-approved treatment for **hyperphagia** in **Prader-Willi syndrome** (PWS).

The paper, titled "**Diazoxide Choline** Extended-Release Tablets in **Prader-Willi Syndrome**: A Randomized, Double-Blind, Placebo-Controlled, Withdrawal Period Study," details results from a 16-week randomized withdrawal study that was a key component of the comprehensive Phase 3 clinical program supporting **VYKAT XR**'s regulatory approval.

Study Design and Participants

The randomized withdrawal period study included 77 individuals who had previously completed 13-week placebo-controlled and long-term open-label Phase 3 studies. Participants were randomized 1:1 to either continue **VYKAT XR** treatment (n=38) or switch to placebo (n=39). The primary endpoint was the change in **Hyperphagia** Questionnaire for Clinical Trials (HQ-CT) total score from baseline to week 16, with secondary endpoints including Clinical Global Impression of Severity (CGI-S) and Improvement (CGI-I) scores.

Key Clinical Findings

The study demonstrated that **hyperphagia** worsened significantly when **VYKAT XR** treatment was withdrawn compared to continued administration. Statistically significant increases in HQ-CT scores from baseline to week 16 were observed with placebo versus VYKAT XR (P=0.0022), indicating worsening of hyperphagia symptoms.

Weight management outcomes also favored continued **VYKAT XR** treatment. The placebo cohort gained more weight and increased their BMI z-score more than the VYKAT XR cohort, with a least squares mean weight difference of -1.6 kg (95% confidence interval: -3.1, -0.1) and a least squares mean z-score difference of -0.09 (-0.17, -0.01).

While CGI-S and CGI-I scores favored **VYKAT XR**, these measures approached but did not reach statistical significance. The safety profile remained consistent with previous studies, with adverse events similar between both treatment arms and no serious adverse events reported in the VYKAT XR treatment arm.

Clinical Significance and Expert Perspectives

"This important study provided additional controlled data confirming the safety and efficacy of **VYKAT XR** in people with PWS," said Dr. Anish Bhatnagar, Chief Executive Officer and Chairman of the Board of **Soleno Therapeutics**. "Our strong momentum since commercial launch reflects both the significant unmet need that VYKAT XR addresses as the first FDA-approved treatment for the hallmark feature of PWS – **hyperphagia** – as well as the meaningful therapeutic benefit that it can offer."

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Dr. Jennifer Miller, Professor of Pediatric Endocrinology at the **University of Florida**, Gainesville, and lead author of the paper, emphasized the clinical importance of the findings. "The compelling results of the randomized withdrawal study highlighted in JCEM further reinforce the meaningful and sustained benefit of **VYKAT XR** in people living with **hyperphagia** caused by PWS," she stated. "These results underscore the critical role that VYKAT XR can play in addressing hyperphagia, the most debilitating and distressing symptom of PWS, where no other effective therapeutic options are currently available."

Comprehensive Clinical Development Program

The published study was part of a comprehensive Phase 3 clinical program that included 127 participants with over 400 patient years of drug exposure, including individuals with nearly six years of continuous treatment. This extensive clinical experience supported **VYKAT XR**'s approval by the **U.S. Food and Drug Administration** on March 26, 2025.

About Prader-Willi Syndrome

Prader-Willi syndrome is a rare genetic neurodevelopmental disorder caused by an abnormality in gene expression on chromosome 15. The **Prader-Willi Syndrome Association USA** estimates that PWS occurs in one in every 15,000 live births. The defining symptom is **hyperphagia**, a chronic and life-threatening condition characterized by intense persistent hunger, food preoccupations, extreme drive to consume food, food-related behavior problems, and lack of normal satiety.

Hyperphagia can lead to significant mortality risks including stomach rupture, choking, and accidental death due to food-seeking behavior, as well as long-term comorbidities such as **diabetes**, **obesity**, and **cardiovascular disease**.

Treatment Indication and Safety Profile

VYKAT XR is indicated for the treatment of **hyperphagia** in adults and pediatric patients 4 years of age and older with **Prader-Willi syndrome**. The drug is contraindicated in patients with known hypersensitivity to **diazoxide**, other components of VYKAT XR, or **thiazides**.

Key safety considerations include monitoring for **hyperglycemia**, including **diabetic ketoacidosis**, and risk of fluid overload with **edema**. The most common adverse reactions with incidence ≥10% and at least 2% greater than placebo included hypertrichosis, edema, hyperglycemia, and rash.

Related Coverage

- FDA Approves First Treatment for Hyperphagia in Prader-Willi Syndrome — Mar 26, 2025
- Rhythm Pharmaceuticals Reports Positive Phase 2 Results for Setmelanotide in Prader-Willi Syndrome — Dec 11, 2025
- Aardvark Therapeutics Initiates International Expansion of Phase 3 HERO Trial for Prader-Willi Syndrome Treatment — Dec 11, 2025
- Soleno Therapeutics Gains FDA Support for DCCR Trial Design in Prader-Willi Syndrome — Jul 5, 2017
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Key Organizations

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Sources

- Soleno Therapeutics Announces Publication of Results from Pivotal Study of VYKAT™ XR in the Journal of Clinical Endocrinology and Metabolism
  finance.yahoo.com Jan 5, 2026
- Soleno Therapeutics Announces Publication of Results from Pivotal Study of VYKAT™ XR in the Journal of Clinical Endocrinology and Metabolism
  markets.businessinsider.com Jan 5, 2026
- Soleno Therapeutics Announces Publication of Positive Study Results for VYKAT XR in ...
  quiverquant.com Jan 5, 2026
- Withdrawal from oral therapy tied to worsened hyperphagia in Prader-Willi syndrome
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