# Diazoxide Choline Extended-Release (DCCR) Tablets Significantly Reduce Hyperphagia in Patients with PWS who are Managed with Strict Food Controls

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Abstract #37
United in HOPE
2025 PWS Conference
June 25-26, 2025
Phoenix, AZ

## INTRODUCTION

### Prader-Willi Syndrome

Prader-Willi syndrome (PWS) is a rare genetic neurobehavioral metabolic disorder characterized by hyperphagia, accumulation of excess fat, hypotonia, and behavioral/ psychological challenges.[1,2] Historically, management of PWS has been limited to strict dietary and environmental controls to restrict food access.

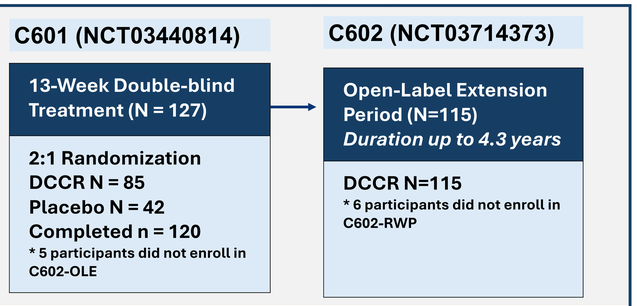
### Diazoxide Choline Extended-Release (DCCR)

DCCR is a once daily, extended-release tablet, which provides for stable plasma concentrations and absorption throughout the GI tract. DCCR tablets have recently been approved by the FDA as VYKAT™ XR for the treatment of hyperphagia in individuals 4 years and over with PWS.[3]

**Study C601** was a Phase 3 randomized (2:1 DCCR to Placebo), double blind, parallel arm study comparing DCCR to Placebo in participants with genetically confirmed PWS, ages 4 and older.

**Study C602** was a Phase 3 multicenter study that consisted of an initial open-label extension (OLE) period for approximately 2 to 4 years **(Figure 1)** followed by a 16-week, double-blind, placebo-controlled randomized withdrawal period.

**Figure 1. C601/C602-OLE Study Design**




## STUDY AIMS

**To evaluate whether DCCR improved hyperphagia in a subset of participants who were highly food-restricted at Baseline based on the "Restrict Food Access" domain of the Food Safe Zone (FSZ) who received DCCR in two Phase 3 studies.**

## METHODS

- Participants were considered highly food-restricted if they scored in the highest quartile (Q4; >9 points) of the "Restrict Food Access" domain at Baseline.
- Hyperphagia was assessed by the Hyperphagia Questionnaire for Clinical Trials (HQ-CT).

## RESULTS

- Of 125 participants ≥4 years old with genetically-confirmed PWS treated with DCCR in the Phase 3 studies, 25 participants scored in the highest quartile of the "Restrict Food Access" domain **(Table 1)**.
- The highly food-restricted group tended to be older than the less restricted group (15.4 vs. 12.8 years), have higher baseline HQ-CT scores (24.6 versus 20.7), and higher BMI-Z (1.8 versus 1.5) **(Table 1)**.

**Table 1. Baseline Characteristics**

| | Less Restricted (≤Q3) (N = 98) | Highly Restricted (>Q3) (N = 25) | Overall (N = 125) |
|---|---|---|---|
| **Age** (mean [±SD]), years | 12.8 (6.3) | 15.4 (9.3) | 13.4 (7.0) |
| **Sex** (% male/female) | 45.9 / 54.1 | 44.0 / 56.0 | 44.8 / 55.2 |
| **Weight** (mean [±SD]), kg | 59.8 (29.0) | 70.9 (34.1) | 62.1 (30.2) |
| **BMI** (mean[±SD]), kg/m2 | 26.6 (8.7) | 31.3 (12.0) | 27.6 (9.6) |
| **BMI z-score** (mean [±SD]), kg/m$^2$ | 1.5 (1.1) | 1.8 (0.9) | 1.5 (1.1) |
| **Hyperphagia (HQ-CT) Total Score** | 20.7 (6.8) | 24.6 (5.0) | 21.5 (6.7) |
| **PWS Subtype** (% Deletion / Non-deletion / NA) | 63.3 / 35.7 / 1.0 | 60.0 / 40.0 / 0 | 61.6 / 37.6 / 0.8 |
| **Ongoing Growth Hormone Use** (% Yes / No) | 84.7 / 15.3 | 76.0 / 24.0 | 82.4 / 17.6 |

Abbreviations: BMI, body mass index; FSZ, Food Safe Zone; HQ-CT, Hyperphagia Questionnaire for Clinical Trials; NA, not available; PWS, Prader-Willi syndrome; SD, standard deviation.
Note: Two participants did not have Baseline FSZ Restrict Food Access Domain Scores.

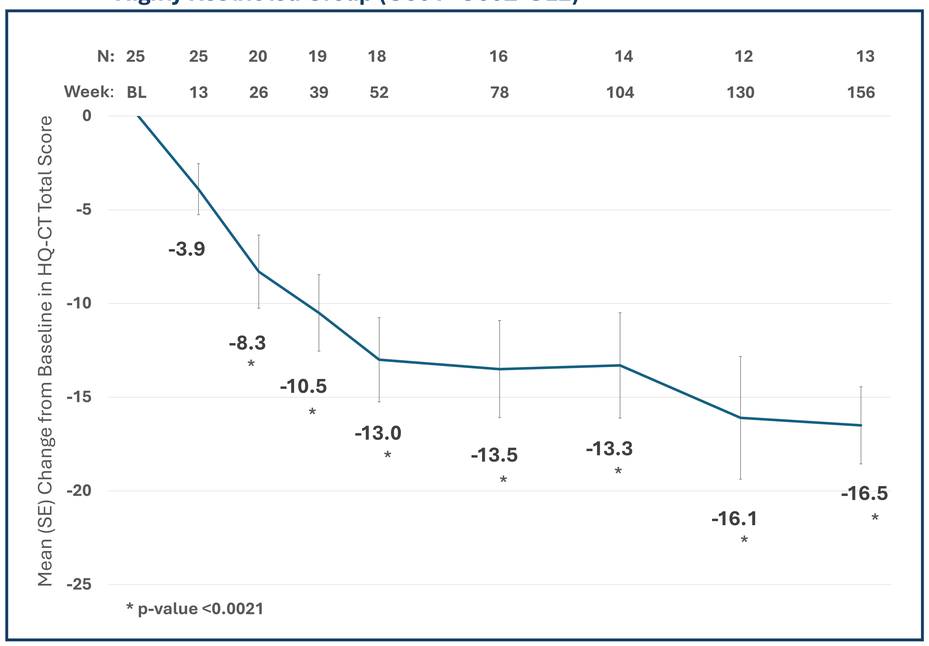
- At Baseline, greater food restriction was associated with higher HQ-CT Total Scores (Pearson correlation [95% CI]: 0.37 (0.20, 0.51); p<0.0001) **(Table 2)**.

**Table 2. C602-OLE Pearson Correlation between Baseline HQ-CT Total Score (0-36) and Baseline Score of Restrict Food Access Domain (0-12)**

| | Less Restricted (≤Q3) (N = 98) | Highly Restricted (>Q3) (N = 25) | Overall (N = 123) |
|---|---|---|---|
| **Pearson Correlation** (95%CI) | 0.31 (0.12, 0.48) | 0.06 (-0.34, 0.45) | **0.37** (0.20, 0.51) |
| **P-value** | 0.0017 | 0.7661 | **<0.0001** |

Abbreviations: CI, confidence interval; HQ-CT, Hyperphagia Questionnaire for Clinical Trials.

**Figure 2. Mean (SE) HQ-CT Total Score (0-36) Change from Baseline – Highly Restricted Group (C601+C602-OLE)**




- Upon treatment, statistically significant, clinically meaningful reductions (improvements) in HQ-CT Total Scores were observed in both groups at all timepoints from Week 26 (p<0.0021) through Year 3 (last assessment).
- In the highly food-restricted group, mean changes were -13.0, -13.3, and -16.5 at Weeks 52, 104, and 156, respectively (**Figure 2**). In the less-restricted group, mean changes were: -10.0, -11.2, and -11.6 at Weeks 52, 104, and 156, respectively.

## CONCLUSIONS

- **Participants with stricter food controls at Baseline tended to have higher HQ-CT Total Scores compared to those with less restrictive food controls.**
- **These findings illustrate the need for new therapeutic options, since restricting food access does not lead to reduced hyperphagia symptoms.**
- **After treatment with DCCR, both groups of participants exhibited statistically significant, clinically meaningful reductions in HQ-CT Total Scores.**
- **Nonetheless, these data demonstrate that regardless of the degree of food restriction at Baseline, participants in either group appear to experience clinical benefit after treatment with DCCR.**

## REFERENCES

1. Butler MG, et al. *Curr Pediatr Rev.* 2019; 15(4):207-244.
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3. VYKAT™ XR [package insert]. Soleno Therapeutics, Inc. 2025.



## ACKNOWLEDGMENTS

Phase 3 program study investigators, participants, and their families. The authors acknowledge Jessica Dronen, MSc, for providing medical writing support.

## CONTACT INFORMATION

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