# DESTINY PWS
# Top-Line Data Call

June 8, 2020 | Soleno Therapeutics

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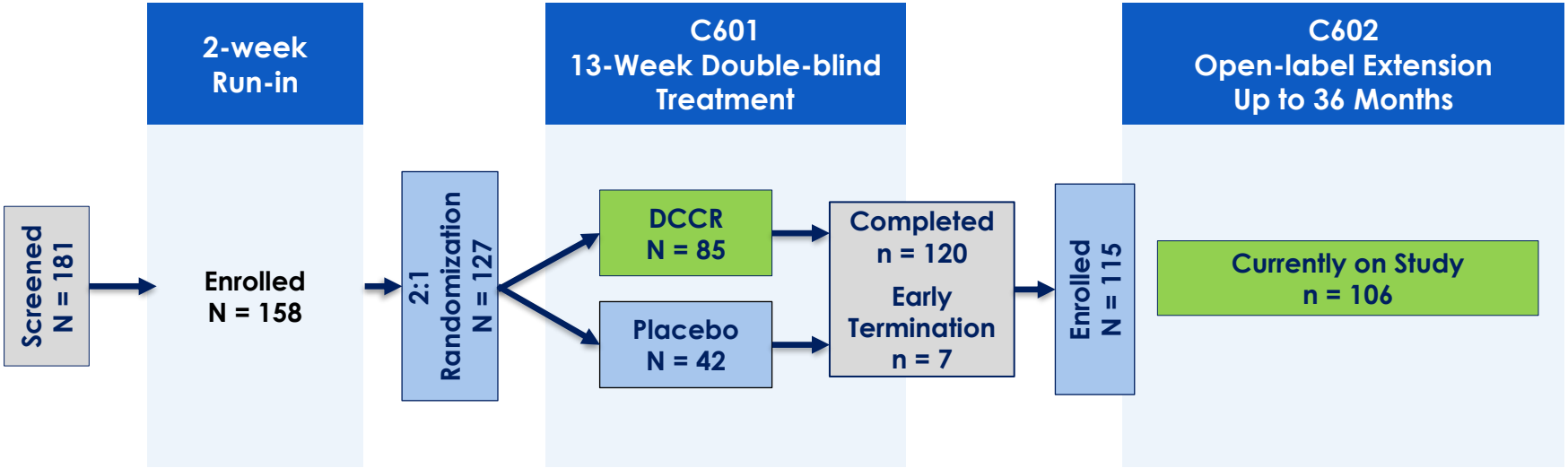
# Phase III Program Design

- C601 (DESTINY PWS): Multi-center, randomized, double-blind, placebo-controlled, parallel arm study in patients with PWS (Phase III)
- C602: Open-label safety extension study





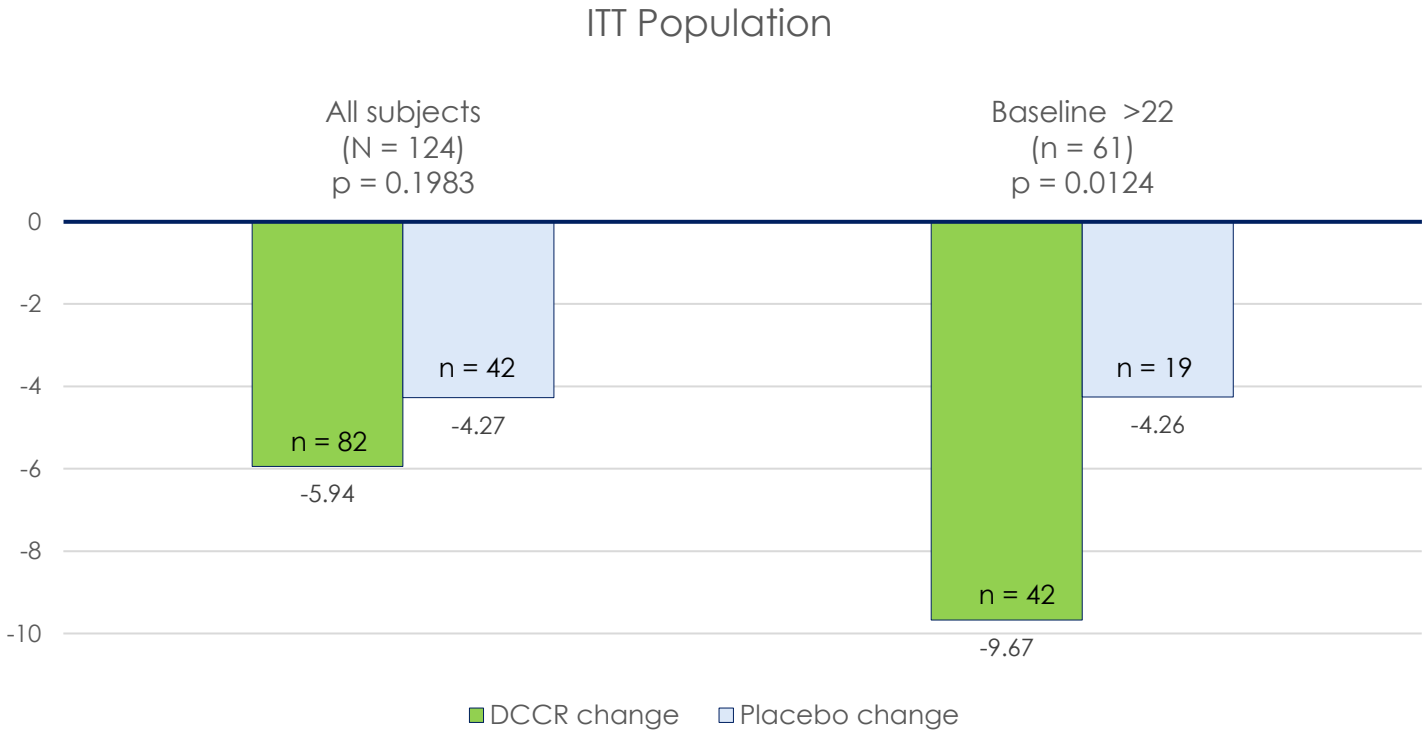
# C601 Demographics and Baseline Characteristics (ITT Population)

| Parameter | DCCR (N = 82) | Placebo (N = 42) | Overall (N = 124) |
|---|---|---|---|
| **Mean Age (years) (SD)** | 13.4 (6.82) | 13.6 (7.37) | 13.5 (6.98) |
| **Gender, n (%)** | | | |
| Male | 36 (43.9) | 19 (45.2) | 55 (44.4) |
| Female | 46 (56.1) | 23 (54.8) | 69 (55.6) |
| **Country, n (%)** | | | |
| United Kingdom | 19 (23.2) | 6 (14.3) | 25 (20.2) |
| United States | 63 (76.8) | 36 (85.7) | 99 (79.8) |
| **PWS Type, n (%)** | | | |
| Deletion | 48 (58.5) | 28 (66.7) | 76 (61.3) |
| Non-deletion | 33 (40.2) | 14 (33.3) | 47 (37.9) |
| Not Available | 1 (1.2) | 0 (0.0) | 1 (0.8) |

# C601 Primary Analysis – Change From Baseline in Hyperphagia to Visit 7

| | DCCR (N = 82) | Placebo (N = 42) |
|---|---|---|
| LS Mean (SE) | -5.94 (0.879) | -4.27 (1.145) |
| 95% CI | (-7.68, -4.20) | (-6.53, -2.00) |
| LS Mean Difference [DCCR-Placebo] (SE) | -1.67 (1.294) | |
| 95% CI | (-4.24, 0.89) | |
| p-value | 0.1983 | |

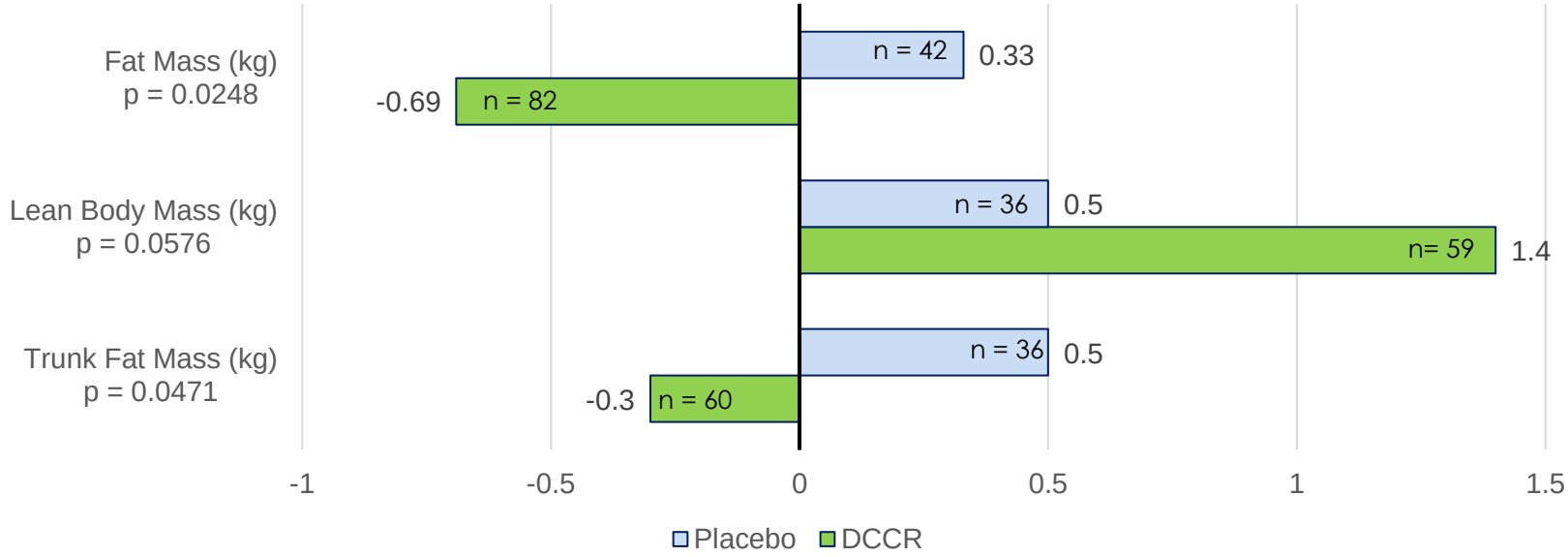
# Changes from Baseline in HQ-CT at Visit 7



# Key Secondary Endpoints

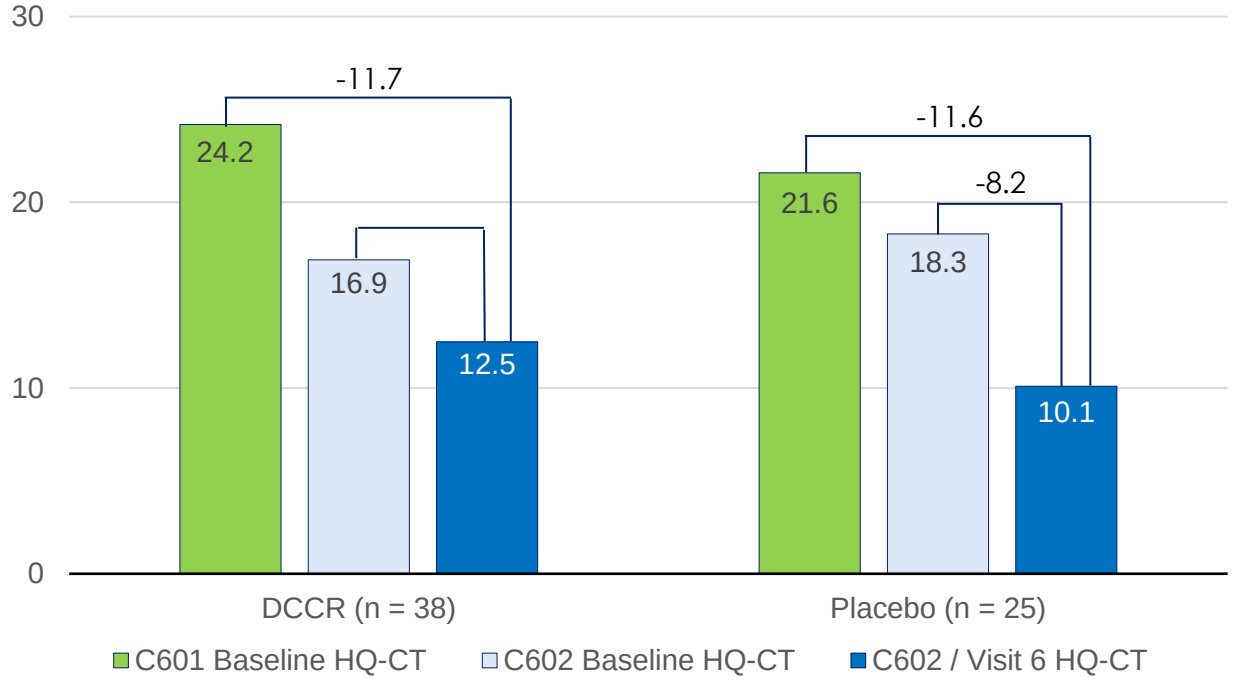
| Endpoint | p-value |
|---|---|
| Clinical Global Impression of Improvement at Visit 7 (CGI-I) | 0.029 |
| Mean Change From Baseline in Body Fat Mass (DXA) | 0.025 |
| Caregiver Global Impression of Change at Visit 7 (CGI-C) | 0.409 |

# C601 Changes in Body Composition




| Endpoint | p-value |
|---|---|
| Lean Body Mass to Fat Mass Ratio | 0.001 |

# Changes in HQ-CT after 13 or 26 Weeks of DCCR Treatment in C601 and C602





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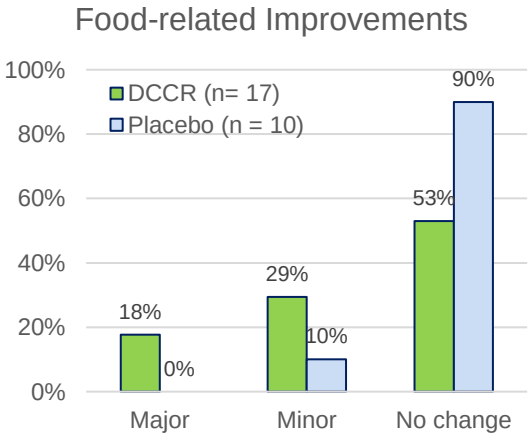
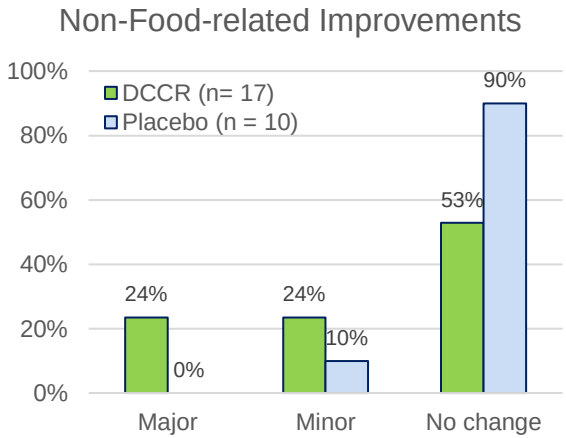
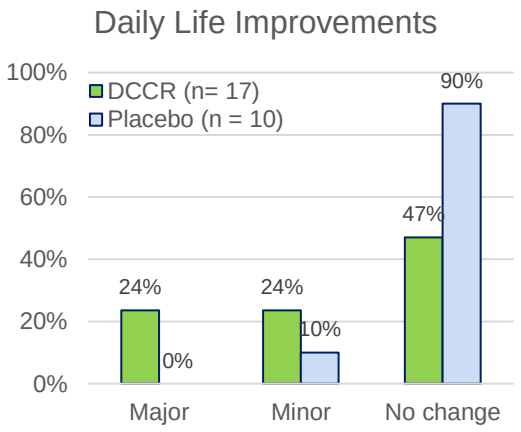
# PWS Outcomes Assessment – Interim Analysis

- *Caregiver interviews at the end of C601*
- *Positive improvements in all 3 domains reported for a significant proportion of DCCR patients (~48%); major improvements reported for 18 - 24% of the patients*
- *Minor improvements reported for one placebo patient (10%); no major improvements were reported for the placebo patients*




*"Minor" includes improvements classified as between major and minor, as well as mixed.*



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# C601 Safety

| Summary of Adverse Events | | |
|---|---|---|
| **Number (%) of subjects with at least one:** | **DCCR (N=84) n (%)** | **Placebo (N=42) n (%)** |
| TEAE | 70 (83.3) | 31 (73.8) |
| TEAE resulting in premature discontinuation of study drug | 4 (4.8) | 1 (2.4) |
| SAE | 6 (7.1) | 0 (0) |
| SAE related to study drug | 1 (1.2) | 0 (0) |
| SAE leading to premature discontinuation of study drug | 2 (2.4) | 0 (0) |

| TEAEs in >5% of DCCR Subjects | | |
|---|---|---|
| **Preferred Term** | **DCCR (N=84) n (%)** | **Placebo (N=42) n (%)** |
| Hypertrichosis | 30 (35.7) | 6 (14.3) |
| Hirsutism | 6 (7.1) | 3 (7.1) |
| Upper Respiratory Tract Infection | 9 (10.7) | 5 (11.9) |
| Edema, peripheral | 17 (20.2) | 4 (9.5) |
| Pyrexia | 5.0 (6) | 0 (0) |
| Headache | 5 (6) | 6 (14.3) |
| Blood glucose increased | 5.0 (6) | 2 (4.8) |
| Hyperglycemia | 10 (11.9) | 0 (0) |

# DCCR Safety Profile

- The safety profile of DCCR in C601 was generally consistent with the known profile of diazoxide and prior experience with DCCR.
- Most events were Grade 1 in severity, including all events of hypertrichosis (except one Grade 2 in the placebo group).
- No Grade 4 or higher events were reported in this study.
- There were no serious unexpected adverse events (SUSARs) related to DCCR.

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