

Supplemental Digital Content 3. Parenteral support at 24 weeks and end of treatment for infants and children based on efficacy population prescription

data

| Parameter | Infants | | | Children* | |
|---|----------------|-------------------------------------|---|-----------------------|--|
| | Core study | | Extension study [†] (N = 2) | Core study (n = 6) | Extension study ^{†§} (n = 6) |
| | SoC (n = 5) | Teduglutide [†] (n = 7) | | | |
| ≥20% reduction in PS volume | | | | | |
| Week 24 | | | | | |
| n | 4 | 6 | - | 6 | - |
| Patients who achieved ≥20% reduction, n (%) | 2 (50.0) | 4 (57.1) | - | 4 (66.7) | - |
| Week 48 | | | | | |
| n | - | - | 2 | - | 5 |
| Patients who achieved ≥20% reduction, n (%) | - | - | 1 (50.0) | - | 4 (80.0) |
| End of treatment | | | | | |
| n | 5 | 7 | 2 | 6 | 5 |
| Patients who achieved ≥20% reduction, n (%) | 3 (60.0) | 4 (57.1) | 1 (50.0) | 4 (66.7) | 4 (80.0) |

Change in PS volume

Baseline

| | | | | | |
|---------------------------------------|-------------|-------------|------------|-------------|-------------|
| n | 5 | 7 | 2 | 6 | 6 |
| Absolute value (mL/kg/day), mean (SD) | 67.7 (13.7) | 94.4 (36.8) | 95.5 (4.5) | 53.9 (30.2) | 53.9 (30.2) |

Week 24

| | | | | | |
|--|--------------|--------------|---|--------------|---|
| n | 4 | 6 | - | 6 | - |
| Absolute change from baseline (mL/kg/day), mean (SD) | -13.3 | -31.3 (15.8) | - | -14.5 (9.2) | - |
| Percentage change from baseline, mean (SD) | -19.6 (18.5) | -36.0 (19.5) | - | -34.6 (34.3) | - |

Week 48

| | | | | | |
|--|---|---|--------------|---|--------------|
| n | - | - | 2 | - | 5 |
| Absolute change from baseline (mL/kg/day), mean (SD) | - | - | -31.3 (26.8) | - | -20.6 (12.3) |
| Percentage change from baseline, mean (SD) | - | - | -32.1 (26.5) | - | -42.5 (38.0) |

End of treatment

| | | | | | |
|---|---|---|---|---|---|
| n | 5 | 7 | 2 | 6 | 5 |
|---|---|---|---|---|---|

| | | | | | |
|---|--------------|--------------|--------------|--------------|--------------|
| Absolute change from baseline (mL/kg/day), mean (SD) | -14.9 (12.3) | -24.1 (24.0) | -31.3 (26.8) | -14.5 (9.2) | -20.6 (12.3) |
| Percentage change from baseline, mean (SD) | -22.4 (17.2) | -27.4 (28.9) | -32.1 (26.5) | -34.6 (34.3) | -42.5 (38.0) |

Change in PS caloric intake

Baseline

| | | | | | |
|---------------------------------------|-------------|-------------|-------------|------------|------------|
| n | 5 | 7 | 2 | 6 | 6 |
| Absolute value (mL/kg/day), mean (SD) | 62.5 (18.3) | 61.8 (15.5) | 50.8 (14.3) | 29.7 (9.8) | 29.7 (9.8) |

Week 24

| | | | | | |
|---|---------------|--------------|---|--------------|---|
| n | 4 | 6 | - | 6 | - |
| Absolute change from baseline (mL/kg/day), mean (SD) | -13.03 (15.1) | -19.7 (9.5) | - | -9.8 (8.7) | - |
| Percentage change from baseline, mean (SD) | -26.1 (32.1) | -34.6 (19.0) | - | -35.3 (33.5) | - |

Week 48

| | | | | | |
|---|---|---|--------------|---|-------------|
| n | - | - | 5 | - | 5 |
| Absolute change from baseline (mL/kg/day), mean (SD) | - | - | -26.4 (13.4) | - | -11.5 (6.6) |

| | | | | | |
|--|--------------|--------------|--------------|--------------|--------------|
| Percentage change from baseline, mean (SD) | - | - | -50.3 (12.2) | - | -44.2 (36.1) |
| End of treatment | | | | | |
| n | 5 | 7 | 5 | 6 | 2 |
| Absolute change from baseline (mL/kg/day), mean (SD) | -20.4 (21.0) | -15.0 (15.2) | -11.5 (6.6) | -9.8 (8.7) | -26.4 (13.4) |
| Percentage change from baseline, mean (SD) | -38.9 (39.9) | -27.4 (25.7) | -44.2 (36.1) | -35.5 (33.5) | -50.3 (12.2) |

End of treatment is the last available measurement during the 24-week treatment period.

*For study NCT02980666 and its extension NCT03268811, six of eight children were enrolled and received teduglutide treatment after protocol amendment, which included improved training for parents to administer doses of teduglutide to patients.

[†]Data pooled from studies NCT03571516 (n = 5; patients randomized to receive teduglutide treatment compared with SoC group) and NCT02980666 (N = 2; patients only received teduglutide).

[‡]Data compared with core study baseline.

[§]Of the children enrolled in the extension study, one child did not receive teduglutide owing to ongoing enteral autonomy from the core study.

PS = parenteral support; SD = standard deviation; SoC = standard of care.