Supplemental Digital Content 7. Z-scores at week 24 and end of treatment for safety populations

Parameter	Infants			Children[*]	
	Core study		Extension study[‡]	Core study	Extension study[‡§]
	SoC	Teduglutide[†]			
Weight-for-age Z-score					
Baseline					
n	5	7	2	8	7
Mean (SD)	−0.6 (1.3)	−1.5 (2.0)	−2.9 (2.7)	−1.9 (1.4)	−1.9 (1.5)
Median (min, max)	0.1 (−2.8, 0.3)	−0.7 (−4.8, 0.8)	−2.9 (−4.8, −1.0)	−1.9 (−3.8, −0.2)	−1.6 (−3.8, −0.2)
Week 24					
N	3	6	-	7	-
Mean (SD)	−1.4 (1.9)	−0.6 (1.1)	-	−1.9 (2.1)	-
Median (min, max)	−0.9 (−3.4, 0.2)	−0.7 (−1.9, 0.7)	-	−1.3 (−5.4, −0.0)	-
Change from baseline, mean (SD)	−0.3 (0.3)	0.4 (1.5)	-	0.0 (0.8)	-
Week 48					
N	-	-	2	-	6

	Col1	Col2	Col3	Col4	Col5
Mean (SD)	-	-	0.4 (0.0)	-	−1.8 (2.0)
Median (min, max)	-	-	0.4 (0.3, 0.4)	-	−1.0 (−4.9, −0.1)
Change from baseline, mean (SD)	-	-	3.3 (2.6)	-	0.0 (0.8)
End of treatment					
n	4	6	2	8	6
Mean (SD)	−0.8 (1.9)	−0.6 (1.1)	0.4 (0.0)	−1.9 (1.9)	−1.8 (2.0)
Median (min, max)	−0.3 (−3.4, 1.0)	−0.7 (−1.9, 0.7)	0.4 (0.3, 0.4)	−1.8 (−5.4, −0.0)	−1.0 (−4.9, −0.1)
Change from baseline, mean (SD)	0.0 (0.6)	0.5 (1.5)	3.3 (2.6)	0.0 (0.8)	0.0 (0.8)
Length/height-for-age Z-score[‖]					
Baseline					
n	5	7	2	8	7
Mean (SD)	0.1 (1.3)	−1.5 (2.3)	−3.7 (1.1)	−2.2 (1.4)	−2.0 (1.4)
Median (min, max)	0.4 (−2.1, 1.2)	−1.2 (−4.5, 1.3)	−3.7 (−4.5, −2.8)	−2.6 (−3.4, 0.5)	−2.5 (−3.3, 0.5)
Week 24					
n	2	6	-	7	-
Mean (SD)	−0.9 (1.9)	−0.9 (1.4)	-	−1.9 (1.4)	-

Median (min, max)	−0.9 (−2.2, 0.5)	−0.2 (−2.9, 0.2)	-	−2.3 (−3.6, 0.4)	-
Change from baseline, mean (SD)	−0.4 (0.4)	0.1 (1.4)	-	0.1 (0.4)	-
Week 48					
n	-	-	2	-	6
Mean (SD)	-	-	−2.3 (0.8)	-	−1.9 (1.7)
Median (min, max)	-	-	−2.3 (−2.9, −1.7)	-	−2.4 (−4.0, 0.4)
Change from baseline, mean (SD)	-	-	1.4 (0.3)	-	0.0 (0.6)
End of treatment					
n	3	6	2	8	6
Mean (SD)	−0.2 (1.8)	−0.9 (1.4)	−2.3 (0.8)	−2.1 (1.5)	−1.9 (1.7)
Median (min, max)	0.5 (−2.2, 1.3)	−0.2 (−2.9, 0.2)	−2.3 (−2.9, −1.7)	−2.5 (−3.6, 0.4)	−2.4 (−2.4, −2.4)
Change from baseline, mean (SD)	0.0 (0.8)	0.1 (1.4)	1.4 (0.3)	0.0 (0.4)	0.0 (0.6)
Weight-for-length Z-score					
Baseline					
n	5	7	2	8	7
Mean (SD)	−0.8 (1.1)	−0.9 (2.3)	−1.3 (4.9)	-	-

Median (min, max)	−0.1 (−2.2, 0.2)	−0.30 (−4.8, 2.1)	−1.3 (−4.8, 2.1)	-	-
Week 24					
n	3	6	-	-	-
Mean (SD)	−1.3 (2.1)	−0.2 (1.6)	-	-	-
Median (min, max)	−1.5 (−3.3, 0.8)	−0.5 (−1.9, 2.4)	-	-	-
Change from baseline, mean (SD)	−0.1 (1.00)	0.4 (2.00)	-	-	-
Week 48					
n	-	-	2	-	-
Mean (SD)	-	-	2.0 (0.6)	-	-
Median (min, max)	-	-	2.0 (1.6, 2.5)	-	-
Change from baseline, mean (SD)	-	-	3.4 (4.3)	-	-
End of treatment					
n	4	6	2		
Mean (SD)	−0.9 (1.9)	−0.2 (1.6)	2.0 (0.6)	-	-
Median (min, max)	−0.5 (−3.3, 0.8)	−0.5 (−1.9, 2.4)	2.0 (1.6, 2.5)	-	-
Change from baseline, mean (SD)	0.1 (0.9)	0.4 (2.0)	3.4 (4.3)	-	-

BMI-for-age Z-score

Baseline

n	5	7	2	8	7
Mean (SD)	-	-	-	−0.9 (1.5)	−1.0 (1.6)
Median (min, max)	-	-	-	−0.8 (−3.6, 1.2)	−1.0 (−3.6, 1.2)

Week 24

n				7	-
Mean (SD)	-	-	-	−0.9 (1.8)	-
Median (min, max)	-	-	-	−0.7 (−3.3, 1.3)	-
Change from baseline, mean (SD)	-	-	-	0.0 (0.8)	-

Week 48

n	-	-	-	-	6
Mean (SD)	-	-	-	-	−0.7 (1.5)
Median (min, max)	-	-	-	-	−0.8 (−2.4, 1.3)
Change from baseline, mean (SD)	-	-	-	-	0.1 (0.7)

End of treatment

	Col1	Col2	Col3	Col4	Col5
n				7	6
Mean (SD)	N/A	N/A	N/A	−0.9 (1.7)	−0.7 (1.5)
Median (min, max)	N/A	N/A	N/A	−0.5 (−3.3, 1.3)	−0.8 (−2.4, 1.3)
Change from baseline, mean (SD)	N/A	N/A	N/A	−0.0 (0.8)	0.1 (0.7)

Head circumference-for-age Z-score

Baseline

	Col1	Col2	Col3	Col4	Col5
n	5	7	2	8	7
Mean (SD)	−0.1 (1.7)	0.5 (1.6)	0.0 (2.8)	2.6 (-)	2.6 (-)
Median (min, max)	−0.4 (−1.7, 2.8)	0.8 (−2.0, 2.1)	0.0 (−2.0, 2.0)	2.6 (2.6, 2.6)	2.6 (2.6, 2.6)

Week 24

	Col1	Col2	Col3	Col4	Col5
n	2	6	-	-	-
Mean (SD)	−1.2 (1.6)	0.7 (1.2)	-	-	-
Median (min, max)	−1.2 (−2.4, −0.0)	0.3 (−0.8, 3.8)	-	-	-
Change from baseline, mean (SD)	−0.2 (0.7)	0.1 (1.1)	-	-	-

Week 48

	Col1	Col2	Col3	Col4	Col5
n	-	-	2	-	-

Mean (SD)	-	-	2.4 (3.46)	-	-
Median (min, max)	-	-	2.4 (−0.0, 4.9)	-	-
Change from baseline, mean (SD)	-	-	2.4 (0.65)	-	-
End of treatment					
n	3	6	2		
Mean (SD)	−1.1 (1.2)	0.7 (1.7)	2.4 (3.5)	N/A	N/A
Median (min, max)	−0.9 (−2.4, −0.0)	0.3 (−0.8, 3.8)	2.4 (−0.0, 4.9)	N/A	N/A
Change from baseline, mean (SD)	−0.3 (0.5)	0.1 (1.1)	2.4 (0.7)	N/A	N/A

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.

[||]Length Z-score calculated for infants and height Z-score recorded for children.

BMI = body mass index; N/A = not applicable; SD = standard deviation; SoC = standard of care.