

## Secondary: Change from baseline in Infant Toddler Quality of Life (ITQoL) scores at Week 52

### Behavior Score Cohort 1:

Table 14.2.13.6.1 Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	≥ 24 to < 36 months (N=4)	≥ 36 to < 60 months (N=12)	Overall (N=16)	≥ 24 to < 36 months (N=9)	≥ 36 to < 60 months (N=10)	Overall (N=19)
ITQoL : Behavior Score (cont.)						
Change from baseline to Week 52						
n	3	10	13	9	8	17
Mean (SD)	14.59 (33.53)	-0.83 (20.42)	2.73 (23.36)	2.55 (11.50)	-2.08 (6.49)	0.37 (9.50)
Median	4.17	0.00	2.08	2.08	-2.08	0.00
25th, 75th Percentile	-12.50, 52.09	-8.33, 14.58	-8.33, 14.58	-2.08, 12.50	-8.33, 1.04	-8.33, 4.17
Min, Max	-12.5, 52.1	-50.0, 18.8	-50.0, 52.1	-18.8, 18.8	-8.3, 10.4	-18.8, 18.8

### Behavior Score Cohort 2:

Table 14.2.13.6.2 Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 2 (≥ 6 to < 24 months)) Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	≥ 6 to < 15 months (N=3)	≥ 15 to < 24 months (N=5)	Overall (N=8)	≥ 6 to < 15 months (N=5)	≥ 15 to < 24 months (N=7)	Overall (N=12)
ITQoL : Behavior Score (cont.)						
Change from baseline to Week 52						
n	0	5	5	1	7	8
Mean (SD)		1.74 (13.60)	1.74 (13.60)	-12.50 (NA)	-2.08 (7.01)	-3.39 (7.46)
Median		0.38	0.38	-12.50	-4.17	-4.17
25th, 75th Percentile		-4.16, 10.41	-4.16, 10.41	-12.50, -12.50	-6.25, 4.17	-8.33, 0.01
Min, Max		-16.7, 18.8	-16.7, 18.8	-12.5, -12.5	-10.4, 10.4	-12.5, 10.4

## Behavior Score Cohort 3:

Table 14.2.13.6.3  
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Behavior Score				
Baseline				
n	1	0	0	1
Mean (SD)	79.17 (NA)			79.17 (NA)
Median	79.17			79.17
25th, 75th Percentile	79.17, 79.17			79.17, 79.17
Min, Max	79.2, 79.2			79.2, 79.2
Week 26				
n	0	1	2	1
Mean (SD)		81.25 (NA)	63.54 (10.31)	81.25 (NA)
Median		81.25	63.54	81.25
25th, 75th Percentile		81.25, 81.25	56.25, 70.83	81.25, 81.25
Min, Max		81.3, 81.3	56.3, 70.8	81.3, 81.3

Table 14.2.13.6.3  
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Sentinel (N=3)	Randomized		All Vosoritide Treated (N=12)
		Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Behavior Score (cont.)				
Week 52				
n	3	8	8	11
Mean (SD)	84.72 (6.37)	83.33 (11.02)	80.21 (18.09)	83.71 (9.67)
Median	83.33	85.42	85.42	83.33
25th, 75th Percentile	79.17, 91.67	75.00, 92.71	65.63, 94.80	77.08, 91.67
Min, Max	79.2, 91.7	64.6, 95.8	50.0, 100.0	64.6, 95.8
Change from baseline to Week 52 <sup>a</sup>				
n	1	0	0	1
Mean (SD)	0.00 (NA)			0.00 (NA)
Median	0.00			0.00
25th, 75th Percentile	0.00, 0.00			0.00, 0.00
Min, Max	0.0, 0.0			0.0, 0.0

## Global Behavior Score Cohort 1:

Table 14.2.13.7.1  
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 ( $\geq 24$  to  $< 60$  months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	$\geq 24$ to $< 36$ months (N=4)	$\geq 36$ to $< 60$ months (N=12)	Overall (N=16)	$\geq 24$ to $< 36$ months (N=9)	$\geq 36$ to $< 60$ months (N=10)	Overall (N=19)

ITQoL : Global Behavior Score (cont.)

Change from baseline to Week 52<sup>a</sup>

n	3	10	13	9	8	17
Mean (SD)	13.33 (36.86)	-2.50 (15.68)	1.15 (21.42)	3.33 (21.21)	-3.13 (15.80)	0.29 (18.58)
Median	0.00	0.00	0.00	0.00	0.00	0.00
25th, 75th Percentile	-15.00, 55.00	0.00, 0.00	0.00, 0.00	0.00, 15.00	0.00, 0.00	0.00, 0.00
Min, Max	-15.0, 55.0	-40.0, 15.0	-40.0, 55.0	-25.0, 40.0	-40.0, 15.0	-40.0, 40.0

## Global Behavior Score Cohort 2:

Table 14.2.13.7.2  
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 2 ( $\geq 6$  to  $< 24$  months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	$\geq 6$ to $< 15$ months (N=3)	$\geq 15$ to $< 24$ months (N=5)	Overall (N=8)	$\geq 6$ to $< 15$ months (N=5)	$\geq 15$ to $< 24$ months (N=7)	Overall (N=12)

ITQoL : Global Behavior Score (cont.)

Change from baseline to Week 52<sup>a</sup>

n	0	5	5	2	7	9
Mean (SD)		3.00 (12.55)	3.00 (12.55)	-12.50 (17.68)	-10.00 (23.98)	-10.56 (21.71)
Median		0.00	0.00	-12.50	0.00	0.00
25th, 75th Percentile		0.00, 15.00	0.00, 15.00	-25.00, 0.00	-30.00, 0.00	-25.00, 0.00
Min, Max		-15.0, 15.0	-15.0, 15.0	-25.0, 0.0	-55.0, 15.0	-55.0, 15.0

### Global Behavior Score Cohort 3:

Table 14.2.13.7.3  
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Global Behavior Score				
Baseline				
n	1	0	0	1
Mean (SD)	100.00 (NA)			100.00 (NA)
Median	100.00			100.00
25th, 75th Percentile	100.00, 100.00			100.00, 100.00
Min, Max	100.0, 100.0			100.0, 100.0
Week 26				
n	0	1	2	1
Mean (SD)		100.00 (NA)	72.50 (17.68)	100.00 (NA)
Median		100.00	72.50	100.00
25th, 75th Percentile		100.00, 100.00	60.00, 85.00	100.00, 100.00
Min, Max		100.0, 100.0	60.0, 85.0	100.0, 100.0

Table 14.2.13.7.3  
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Global Behavior Score (cont.)				
Week 52				
n	3	8	8	11
Mean (SD)	90.00 (8.66)	87.50 (24.20)	91.25 (14.33)	88.18 (20.65)
Median	85.00	100.00	100.00	100.00
25th, 75th Percentile	85.00, 100.00	85.00, 100.00	85.00, 100.00	85.00, 100.00
Min, Max	85.0, 100.0	30.0, 100.0	60.0, 100.0	30.0, 100.0
Change from baseline to Week 52				
n	1	0	0	1
Mean (SD)	-15.00 (NA)			-15.00 (NA)
Median	-15.00			-15.00
25th, 75th Percentile	-15.00, -15.00			-15.00, -15.00
Min, Max	-15.0, -15.0			-15.0, -15.0

## Getting On with Other Score Cohort 1:

Table 14.2.13.8.1 Infant Toddler Quality of Life (ITQoL): Getting On With Others Score (Cohort 1 (>= 24 to < 60 months)) Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	>= 24 to < 36 months (N=4)	>= 36 to < 60 months (N=12)	Overall (N=16)	>= 24 to < 36 months (N=9)	>= 36 to < 60 months (N=10)	Overall (N=19)
ITQoL : Getting on With Others Score (cont.)						
Change from baseline to Week 52 <sup>a</sup>						
n	3	9	12	9	8	17
Mean (SD)	17.22 (27.75)	4.63 (12.98)	7.78 (17.18)	0.37 (9.31)	3.29 (8.71)	1.74 (8.88)
Median	8.33	3.33	4.17	1.67	0.00	0.00
25th, 75th Percentile	-5.00, 48.33	3.33, 6.67	0.00, 11.67	-6.67, 3.34	-2.50, 7.50	-5.00, 6.67
Min, Max	-5.0, 48.3	-20.0, 28.3	-20.0, 48.3	-13.3, 16.7	-5.0, 21.3	-13.3, 21.3

## Getting On with Other Score Cohort 2:

Table 14.2.13.8.2 Infant Toddler Quality of Life (ITQoL): Getting On With Others Score (Cohort 2 (>= 6 to < 24 months)) Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	>= 6 to < 15 months (N=3)	>= 15 to < 24 months (N=5)	Overall (N=8)	>= 6 to < 15 months (N=5)	>= 15 to < 24 months (N=7)	Overall (N=12)
ITQoL : Getting on With Others Score (cont.)						
Change from baseline to Week 52 <sup>a</sup>						
n	0	5	5	1	7	8
Mean (SD)		2.33 (7.22)	2.33 (7.22)	-1.79 (NA)	1.19 (5.67)	0.82 (5.35)
Median		3.33	3.33	-1.79	1.67	1.67
25th, 75th Percentile		-3.33, 3.34	-3.33, 3.34	-1.79, -1.79	-3.33, 6.67	-2.56, 4.17
Min, Max		-5.0, 13.3	-5.0, 13.3	-1.8, -1.8	-8.3, 8.3	-8.3, 8.3

### Getting On with Other Score Cohort 3:

Table 14.2.13.8.3  
Infant Toddler Quality of Life (ITQoL): Getting On With Others Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Getting on With Others Score				
Week 26				
n	0	1	2	1
Mean (SD)		78.57 (NA)	66.67 (16.50)	78.57 (NA)
Median		78.57	66.67	78.57
25th, 75th Percentile		78.57, 78.57	55.00, 78.33	78.57, 78.57
Min, Max		78.6, 78.6	55.0, 78.3	78.6, 78.6
Week 52				
n	3	8	8	11
Mean (SD)	78.33 (3.34)	74.24 (11.72)	75.84 (8.54)	75.36 (10.10)
Median	78.33	76.67	77.50	78.33
25th, 75th Percentile	75.00, 81.67	65.00, 82.80	69.17, 83.34	66.67, 81.67
Min, Max	75.0, 81.7	55.0, 90.0	61.7, 85.0	55.0, 90.0

## Change in Health Score Cohort 1:

Table 14.2.13.10.1						
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 ( $\geq 24$ to $< 60$ months))						
Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	$\geq 24$ to $< 36$ months (N=4)	$\geq 36$ to $< 60$ months (N=12)	Overall (N=16)	$\geq 24$ to $< 36$ months (N=9)	$\geq 36$ to $< 60$ months (N=10)	Overall (N=19)
ITQoL : Change in Health Score (cont.)						
Change from baseline to Week 52 <sup>a</sup>						
n	3	10	13	8	8	16
Mean (SD)	0.00 (0.00)	0.10 (0.74)	0.08 (0.64)	0.00 (0.53)	0.25 (0.71)	0.13 (0.62)
Median	0.00	0.00	0.00	0.00	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 1.00	0.00, 0.00	0.00, 0.00	0.00, 1.00	0.00, 0.50
Min, Max	0.0, 0.0	-1.0, 1.0	-1.0, 1.0	-1.0, 1.0	-1.0, 1.0	-1.0, 1.0

## Change in Health Score Cohort 2:

Table 14.2.13.10.2						
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 2 ( $\geq 6$ to $< 24$ months))						
Analysis Population: Full Analysis Set						
Score Visit Results	Randomized Placebo			All Vosoritide Treated		
	$\geq 6$ to $< 15$ months (N=3)	$\geq 15$ to $< 24$ months (N=5)	Overall (N=8)	$\geq 6$ to $< 15$ months (N=5)	$\geq 15$ to $< 24$ months (N=7)	Overall (N=12)
ITQoL : Change in Health Score (cont.)						
Change from baseline to Week 52 <sup>a</sup>						
n	0	5	5	1	5	6
Mean (SD)		-0.60 (1.34)	-0.60 (1.34)	1.00 (NA)	0.20 (0.45)	0.33 (0.52)
Median		0.00	0.00	1.00	0.00	0.00
25th, 75th Percentile		-2.00, 0.00	-2.00, 0.00	1.00, 1.00	0.00, 0.00	0.00, 1.00
Min, Max		-2.0, 1.0	-2.0, 1.0	1.0, 1.0	0.0, 1.0	0.0, 1.0

## Change in Health Score Cohort 3:

Table 14.2.13.10.3  
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Change in Health Score				
Baseline				
n	0	1	1	1
Mean (SD)		3.00 (NA)	5.00 (NA)	3.00 (NA)
Median		3.00	5.00	3.00
25th, 75th Percentile		3.00, 3.00	5.00, 5.00	3.00, 3.00
Min, Max		3.0, 3.0	5.0, 5.0	3.0, 3.0
Week 26				
n	0	2	0	2
Mean (SD)		3.00 (0.00)		3.00 (0.00)
Median		3.00		3.00
25th, 75th Percentile		3.00, 3.00		3.00, 3.00
Min, Max		3.0, 3.0		3.0, 3.0

Table 14.2.13.10.3  
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 3 (0 to < 6 months))  
Analysis Population: Full Analysis Set

Score Visit Results	Randomized			All Vosoritide Treated (N=12)
	Sentinel (N=3)	Vosoritide (N=9)	Placebo (N=8)	
ITQoL : Change in Health Score (cont.)				
Week 52				
n	3	8	7	11
Mean (SD)	3.00 (0.00)	3.25 (0.71)	3.43 (0.53)	3.18 (0.60)
Median	3.00	3.00	3.00	3.00
25th, 75th Percentile	3.00, 3.00	3.00, 3.00	3.00, 4.00	3.00, 3.00
Min, Max	3.0, 3.0	3.0, 5.0	3.0, 4.0	3.0, 5.0
Change from baseline to Week 52				
n	0	1	0	1
Mean (SD)		0.00 (NA)		0.00 (NA)
Median		0.00		0.00
25th, 75th Percentile		0.00, 0.00		0.00, 0.00
Min, Max		0.0, 0.0		0.0, 0.0