

Secondary Endpoint:

Weight compared to the 2006 World Health Organization (WHO) Growth Standards at each timepoint.

Characteristic	Visit/ Timepoint	Statistics	Boys (N=17)		Girls (N=17)	
			Absolute	Change from Baseline	Absolute	Change from Baseline
		P5; P95	-0.84; 2.11	0.06; 2.48	-0.07; 1.37	-0.48; 1.57
		Min; Max	-0.8; 2.1	0.1; 2.5	-0.1; 1.4	-0.5; 1.6
	Month 60	n (missing)	8 (8)	8 (8)	8 (8)	8 (8)
		Mean±SD	0.50±1.119	0.96±0.795	0.61±0.417	0.16±0.570
		Median	0.56	0.84	0.59	0.13
		Q1; Q3	-0.45; 1.39	0.33; 1.39	0.27; 0.90	-0.33; 0.45
		P5; P95	-1.08; 2.06	0.11; 2.43	0.07; 1.29	-0.45; 1.23
		Min; Max	-1.1; 2.1	0.1; 2.4	0.1; 1.3	-0.5; 1.2
	Month 66	n (missing)	7 (9)	7 (9)	7 (9)	7 (9)
		Mean±SD	0.46±0.811	0.71±0.466	1.19±1.046	0.48±0.666
		Median	0.64	0.43	0.86	0.45
		Q1; Q3	-0.32; 0.97	0.33; 1.10	0.66; 1.23	0.01; 0.94
		P5; P95	-0.76; 1.66	0.26; 1.44	0.42; 3.50	-0.51; 1.59
		Min; Max	-0.8; 1.7	0.3; 1.4	0.4; 3.5	-0.5; 1.6
Weight SDS	Month 72	n (missing)	0 (16)	0 (16)	0 (16)	0 (16)
	Month 78	n (missing)	0 (16)	0 (16)	0 (16)	0 (16)
	Month 84	n (missing)	0 (16)	0 (16)	0 (16)	0 (16)
	End of Treatment	n (missing)	17 (0)	17 (0)	17 (0)	17 (0)
		Mean±SD	1.01±1.060	0.60±0.684	0.82±1.112	0.22±0.588
		Median	0.97	0.43	0.86	0.09
		Q1; Q3	0.32; 1.66	0.26; 0.96	0.29; 1.16	-0.01; 0.58
		P5; P95	-0.76; 3.29	-0.30; 2.43	-1.76; 3.50	-0.71; 1.59
		Min; Max	-0.8; 3.3	-0.3; 2.4	-1.8; 3.5	-0.7; 1.6

Monthly blood Phe levels and Phe levels on the day of the IQ test

Visit/ Timepoint	Statistics	Naïve Subjects (N=11)		Previously Treated (N=23)		Overall (N=34)	
		Absolute	Change from Baseline	Absolute	Change from Baseline	Absolute	Change from Baseline
Baseline	N (missing)	10 (1)		23 (0)		33 (1)	
	Mean ±SD	371.68±188.509		277.57±129.988		306.09±153.427	
	Median	330.10		264.30		270.70	
	Q1; Q3	252.30; 441.10		182.40; 358.10		209.30; 395.60	
	Min; Max	171.5; 825.0		50.0; 559.8		50.0; 825.0	
Month 1	N (missing)	11 (0)	10 (1)	0	0	11 (0)	10 (1)
	Mean ±SD	220.13±114.185	-146.62±196.329			220.13±114.185	-146.62±196.329
	Median	194.15	-104.69			194.15	-104.69
	Q1; Q3	133.08; 314.93	-125.53; -62.40			133.08; 314.93	-125.53; -62.40
	Min; Max	76.8; 484.5	-691.9; 2.4			76.8; 484.5	-691.9; 2.4
Month 3	N (missing)	11 (0)	10 (1)	22 (1)	22 (1)	33 (1)	32 (2)
	Mean ±SD	252.72±99.698	-109.84±156.693	271.59±74.573	-12.99±108.397	265.30±82.688	-43.26±131.026
	Median	289.65	-104.22	291.68	-11.90	289.65	-40.92
	Q1; Q3	161.52; 333.88	-151.40; -91.27	219.18; 325.30	-73.93; 72.45	208.97; 325.30	-113.83; 61.99
	Min; Max	100.9; 390.7	-471.5; 120.4	123.7; 404.8	-228.7; 166.0	100.9; 404.8	-471.5; 166.0

		Naïve Subjects (N=11)		Previously Treated (N=23)		Overall (N=34)	
Visit/ Timepoint	Statistics	Absolute	Change from Baseline	Absolute	Change from Baseline	Absolute	Change from Baseline
End of Treatment	N (missing)	2 (0)	2 (0)	0	0	2 (0)	2 (0)
	Mean ±SD	302.69±39.303	-158.86±10.383			302.69±39.303	-158.86±10.383
	Median	302.69	-158.86			302.69	-158.86
	Q1; Q3	274.90; 330.48	-166.20; - 151.52			274.90; 330.48	-166.20; - 151.52
	Min; Max	274.9; 330.5	-166.2; -151.5			274.9; 330.5	-166.2; -151.5
Safety Follow-up	N (missing)	1 (1)	1 (1)	0	0	1 (1)	1 (1)
	Mean ±SD	305.20	-135.90			305.20	-135.90
	Median	305.20	-135.90			305.20	-135.90
	Q1; Q3	305.20; 305.20	-135.90; - 135.90			305.20; 305.20	-135.90; - 135.90
	Min; Max	305.2; 305.2	-135.9; -135.9			305.2; 305.2	-135.9; -135.9