

Table 14.2.8.4 Summary of Leiter Scale (Total Equivalence Age AM) - by timepoint - FAS

Parameter Timepoint	Actual value	Absolute change from baseline	% Change from baseline	Change from baseline	t-Test results		
					t-value (df)	p-value	95% CI
Total Equivalence Age AM (Years)							
Baseline							
n	24						
Mean (SD)	6.514 (2.176)						
Median	7.083						
Min; Max	2.25; 10.17						
Month 6							
n	15	15	15	Absolute change	0.291 (14)	0.775	(-0.637, 0.837)
Mean (SD)	6.400 (2.424)	0.100 (1.331)	5.219 (22.135)				
Median	6.417	0.167	4.274	% Change	0.913 (14)	0.377	(-7.039,17.477)
Min; Max	2.42; 10.17	-2.83; 2.42	-34.34; 50.00				
Month 12							
n	22	22	22	Absolute change	0.624 (21)	0.540	(-0.389, 0.722)
Mean (SD)	6.860 (1.992)	0.167 (1.254)	5.849 (19.657)				
Median	6.833	0.125	1.515	% Change	1.396 (21)	0.177	(-2.866,14.565)
Min; Max	2.25; 9.75	-2.58; 2.00	-31.31; 48.57				
Month 18							
n	2	2	2	Absolute change	-0.750 (1)	0.590	(-13.46,11.956)
Mean (SD)	3.792 (2.180)	-0.750 (1.414)	-19.42 (34.413)				
Median	3.792	-0.750	-19.42	% Change	-0.798 (1)	0.571	(-328.6,289.78)
Min; Max	2.25; 5.33	-1.75; 0.25	-43.75; 4.92				
Month 24							
n	10	10	10	Absolute change	0.206 (9)	0.842	(-1.083, 1.300)
Mean (SD)	6.817 (1.529)	0.108 (1.665)	11.244 (33.786)				
Median	6.833	0.333	4.848	% Change	1.052 (9)	0.320	(-12.93,35.414)
Min; Max	4.17; 9.08	-2.92; 2.17	-35.35; 85.19				

Note 1: FAS=Full Analysis Set. n=Number of subjects with data.

Note 2: Data for rhLAMANO5 subjects only.

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Parameter Timepoint	Actual value	Absolute change from baseline	% Change from baseline	t-Test results			
				Change from baseline	t-value (df)	p-value	95% CI
Total Equivalence Age AM							
(Years) Month 36							
n	4	4	4	Absolute change	0.899 (3)	0.435	(-2.118, 3.785)
Mean (SD)	5.250 (0.561)	0.833 (1.855)	33.225(47.595)				
Median	5.500	1.208	32.944	% Change	1.396 (3)	0.257	(-42.51,108.96)
Min; Max	4.42; 5.58	-1.75; 2.67	-24.42; 91.43				
Last Observation							
n	24	24	24	Absolute change	0.504 (23)	0.619	(-0.485, 0.798)
Mean (SD)	6.670 (1.757)	0.156 (1.519)	9.345(32.485)				
Median	6.833	0.250	3.974	% Change	1.409 (23)	0.172	(-4.372,23.063)
Min; Max	2.25; 9.75	-2.92; 2.67	-43.75; 91.43				

Note 1: FAS=Full Analysis Set. n=Number of subjects
with data. Note 2: Data for rhLAMANO5 subjects only.