

Table 14.2.18.1 Summary of EQ-5D-L index value - 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
EQ-5D-5L Index							
Baseline							
n	24						
Mean (SD)	0.6217(0.1698)						
Median	0.6120						
Min; Max	0.268; 1.000						
Month 6							
n	14	14	14	Absolute change	2.0196 (13)	0.065	(-0.0045, 0.1339)
Mean (SD)	0.6596(0.1492)	0.0647(0.1199)	17.2811(32.8088)	% Change	1.9708 (13)	0.070	(-1.6621,36.2243)
Median	0.6080	0.0390	6.4501				
Min; Max	0.465; 1.000	-0.102; 0.277	-17.989;103.358				
Month 12							
n	21	21	21	Absolute change	1.5171 (20)	0.145	(-0.0130, 0.0821)
Mean (SD)	0.6678(0.1785)	0.0346(0.1044)	6.9320(19.0980)	% Change	1.6633 (20)	0.112	(-1.7613,15.6253)
Median	0.6420	0.0000	0.0000				
Min; Max	0.275; 1.000	-0.137; 0.264	-21.373; 63.200				
Month 18							
n	2	2	2	Absolute change	2.2159 (1)	0.270	(-0.9231, 1.3131)
Mean (SD)	0.6385(0.1181)	0.1950(0.1245)	44.1743(28.6949)	% Change	2.1771 (1)	0.274	(-213.64,301.988)
Median	0.6385	0.1950	44.1743				
Min; Max	0.555; 0.722	0.107; 0.283	23.884; 64.465				
Month 24							
n	10	10	10	Absolute change	0.6359 (9)	0.541	(-0.0670, 0.1194)
Mean (SD)	0.6437(0.2057)	0.0262(0.1303)	7.2199(21.9332)	% Change	1.0409 (9)	0.325	(-8.4702,22.9099)
Median	0.5670	0.0225	5.2417				
Min; Max	0.279; 1.000	-0.260; 0.180	-31.439; 48.000				

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

Note 2: Data for rhLAMANO5 subjects only.

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				Change from baseline	t-value (df)	p-value	95% CI
EQ-5D-5L Index							
Month 36							
n	4	4	4	Absolute change	1.3962 (3)	0.257	(-0.1270, 0.3255)
Mean (SD)	0.7158(0.0743)	0.0993(0.1422)	21.1495(32.1006)				
Median	0.7040	0.0475	8.3664	% Change	1.3177 (3)	0.279	(-29.930,72.2287)
Min; Max	0.649; 0.806	0.000; 0.302	0.000; 67.865				
Last Observation							
n	24	24	24	Absolute change	1.8299 (23)	0.080	(-0.0066, 0.1075)
Mean (SD)	0.6722(0.1674)	0.0505(0.1351)	11.2291(24.7218)				
Median	0.6550	0.0225	5.2417	% Change	2.2252 (23)	0.036	(0.7900,21.6682)
Min; Max	0.279; 1.000	-0.260; 0.302	-31.439; 67.865				

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