

Supplementary material

Treatment satisfaction in 5q-spinal muscular atrophy under nusinersen therapy

Short title ***Treatment satisfaction in 5q-SMA***

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Supplementary table 1: Univariate regression results: analysis of relevant clinical features with influence on the TSQM-1.4[©]

dimensions and sum score.

	Global satisfaction		Effectiveness		Side effects		Convenience		Sum score	
	P-value	β [95%-CI]	P-value	β [95%-CI]	P-value	β [95%-CI]	P-value	β [95%-CI]	P-value	β [95%-CI]
Age	0.885	-0.03 [-0.37-0.32]	0.653	-0.08 [-0.42-0.27]	0.858	-0.02 [-0.26-0.22]	0.194	-0.22 [-0.55- 0.11]	0.535	-0.29 [-1.21- 0,63]
Sex, female	0.503	3.01 [-6.03-12.20]	0.208	5.78 [-3.28-14.83]	0.677	-1.35 [-7.78-5.07]	0.471	-3.25 [-12.18- 5.68]	0.659	5.46 [-19.04- 29,95]
SMA type	0.344	-3.64 [-11.24-3.96]	0.905	-0.46 [-8.08-7.16]	0.459	-2.01 [-7.36-3.35]	0.248	4.31 [-3.06- 11.68]	0.992	0.10 [-20.19- 20,40]
SMN2 copy number n=79	0.294	-3.25 [-9.37-2.87]	0.822	0.67 [-5.26-6.61]	0.082	-3.90 [-8.30-0.50]	0.032	6.38 [0.57- 12.19]	0.928	0.77 [-16.10- 17,64]
Age at onset	0.282	0.35 [-0.29-0.99]	0.045	0.64 [0.01-1.26]	0.326	-0.23 [-0.68-0.23]	0.138	0.47 [-0.16- 1.10]	0.090	1.26 [-0.24- 3,15]
Disease duration	0.479	-0.12 [-0.46-0.22]	0.130	-0.26 [-0.60-0.08]	0.737	0.04 [-0.20-0.28]	0.037	-0.35 [-0.67- -0.02]	0.127	-0.70 [-1.61- 0.20]
Ambulatory state	0.093	8.19 [-1.38-17.76]	0.015	11.57 [2.27-20.87]	0.336	-3.32 [-10.12-3.49]	0.003	14.28 [4.96- 23.60]	0.005	36.96 [11.69- 62.23]
RULM* n=78	0.869	-0.03 [-0.39-0.33]	0.137	0.27 [-0.09-0.63]	0.037	-0.27 [-0.52- -0.02]	0.106	0.28 [-0.06- 0.62]	0.406	0.41 [-0.57- 1.38]
HFMSE*n=78	0.304	0.11 [-0.11-0.33]	0.023	0.25 [0.04-0.46]	0.017	-0.19 [-0.34-0.04]	0.160	0.15 [-0.06- 0.36]	0.083	0.402 [-0.07- 1.05]
CT-guided LP	0.859	-0.79 [-9.58-8.00]	0.962	0.21 [-8.61-9.03]	0.112	4.93 [-1.17-11.03]	0.002	-12.88 [-21.04- -4.72]	0.176	-10.63 [-0.19- 0.99]

CT=computed tomography, HFMSE=Hammersmith Functional Motor Scale Expanded, LP=lumbar puncture, n=number, RULM=Revised Upper Limb Module, SMA=spinal muscular atrophy, SMN2=survival of motor neuron 2, 95%-CI=95% confidence interval, β= beta coefficient (the degree of change in the outcome variable for every 1-unit of change in the variable). *RULM and HFMSE scores at treatment initiation. p<0.2 marked in bold.