Real-world clinical experience with long-term miglustat maintenance therapy in type 1 Gaucher disease: the ZAGAL project

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Online Supplementary Table A, B,C. Mean percentage change from baseline in standard disease parameters at 36 months.

A)

Hb g/dL	N	Mean	SD	MD	95%	CI	Plat x10⁹/L Mean	SD	MD	95%	CI
Baseline	28	13.5	1.6				161.6	70.5			
Month 6	28	13.6	1.3	0.1	-0.2	0.4	160.6	62.6	-1.0	-15.7	13.7
Baseline	28	14.1	1.7				157.3	67.7			
Month 12	28	13.9	1.2	-0.1	-0.6	0.4	149.1	55.8	-8.2	-22.7	6.3
Baseline	22	14.0	1.9				145.8	44.5			
Month 24	22	13.9	1.1	-0.1	-0.9	0.8	148.2	40.6	2.3	-14.5	19.2
Baseline	18	13.6	1.6				145.3	52.9			
Month 36	18	13.6	0.6	0.0	-1.0	1.0	143.2	43.1	-2.1	-27.4	23.2
Baseline	9	13.8	0.9				165.7	51.0			
Month 48	9	13.5	0.8	-0.3	-2.5	2.0	148.0	33.3	-17.7	-67.6	32.3
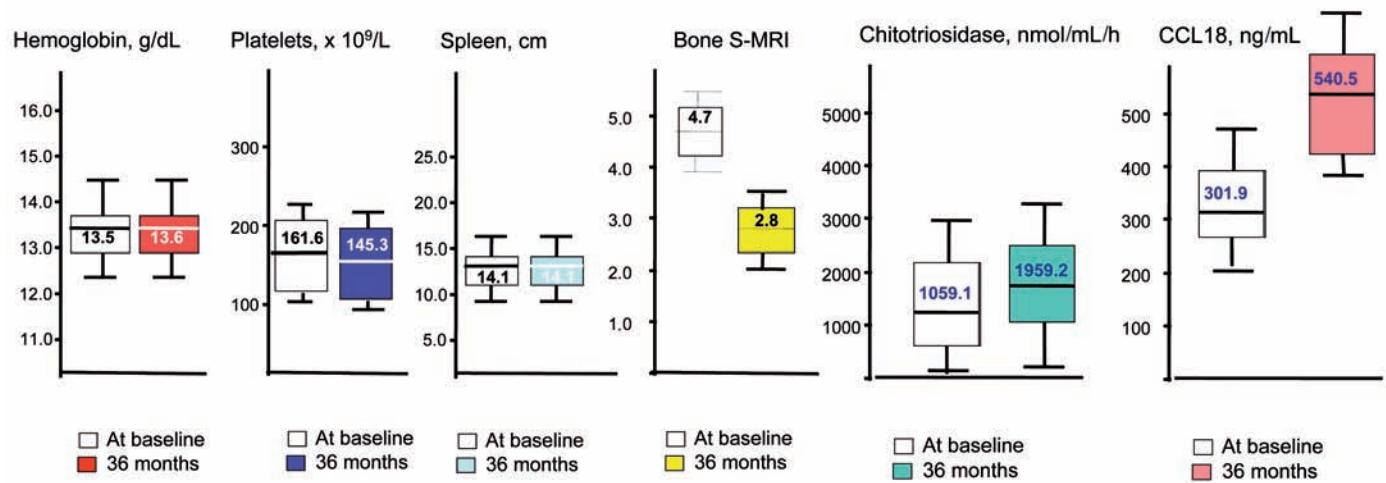
MD: Mean difference from baseline

B)
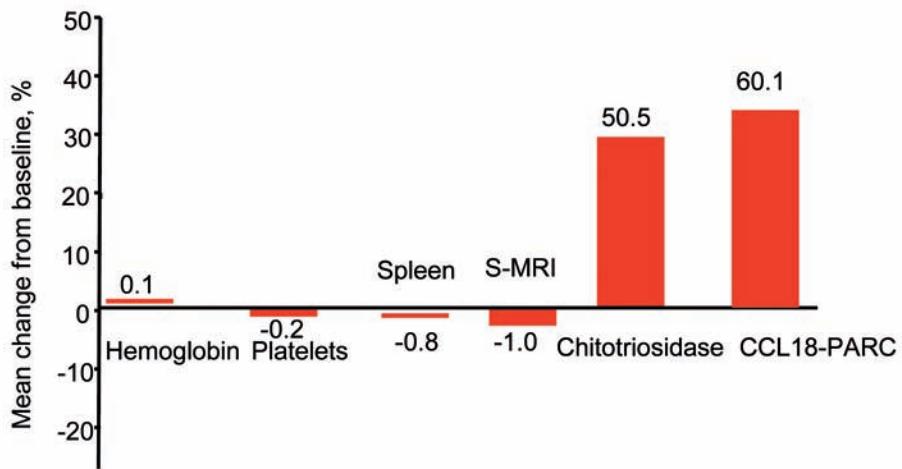
Spleen length (cm)	N	Mean	SD	MD	95%	CI	Bone MRI score (units) Mean	SD	MD	95%	CI
Baseline	20	14.1	1.5				4.7	5.2			
Month 6	20	13.9	1.4	-0.2	-0.8	0.4	4.4	4.8	-0.3	-0.7	0.2
Baseline	20	14.2	1.6				4.8	5.8			
Month 12	20	13.7	1.0	-0.5	-1.1	0.1	3.9	5.2	-0.9	-1.8	0.0
Baseline	14	14.1	1.5				3.6	3.0			
Month 24	14	13.6	1.1	-0.5	-1.2	0.2	2.8	2.4	-0.9	-1.7	0.0
Baseline	10	14.1	1.4				2.8	2.4			
Month 36	10	13.4	0.7	-0.8	-1.7	0.2	1.8	1.0	-1.0	-4.2	2.2
Baseline	5	13.3	0.6				1.3	1.5			
Month 48	5	13.3	0.6	-	-	-	1.3	1.5	-	-	-

C)

Chitotriosidase nM/mL/h	N	Mean	SD	MD	95%	CI	PARC ng/mL Mean	SD	MD	95%	CI
Baseline	28	1059.1	1079.0				301.9	267.8			
Month 6	28	1075.4	1002.9	16.3	-467.1	499.8	261.6	147.0	-40.3	-132.0	51.5
Baseline	28	1043.1	797.9				302.8	297.8			
Month 12	28	1250.8	1490.4	207.7	-414.0	829.5	328.1	298.1	25.4	-48.2	98.9
Baseline	22	778.3	456.3				254.0	327.4			
Month 24	22	1180.6	835.1	402.4	23.2	781.5	371.6	308.0	117.6	-91.3	326.5
Baseline	18	1300.8	1236.8				335.2	339.7			
Month 36	18	1959.2	1487.1	658.4	-348.2	1665.0	540.5	355.3	205.3	-81.4	492.0
Baseline	9	456.3	253.2				121.0	21.9			
Month 48	9	854.3	711.7	398.0	-337.0	1133.0	368.7	286.6	247.7	-411.9	907.2

Online Supplementary Figure S1A. Changes from baseline in standard disease parameters at 48 months.

Online Supplementary Figure S1B. Mean % changes from baseline in standard disease parameters at 48 months.