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Parker, Helen, Ellison, Stuart M, Holley, Rebecca J, O'Leary, Claire, Liao, Aiyin, Asadi, Jalal, Glover, Emily, Ghosh, Arunabha, Jones, Simon, Wilkinson, Fiona L , Brough, David , Pinteaux, Emmanuel, Boutin, Hervé and Bigger, Brian W (2020) Haematopoietic stem cell gene therapy with IL1Ra rescues cognitive loss in mucopolysaccharidosis IIIA. EMBO Molecular Medicine, 12 (3). e11185 ISSN 1757-4684

DOI: https://doi.org/10.15252/emmm.201911185

Publisher: Nature Publishing Group UK

Version: Supplemental Material

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Additional Information: This is an open access article which first appeared in EMBO Molecular Medicine

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Haematopoietic stem cell gene therapy with IL-1Ra rescues cognitive loss in mucopolysaccharidosis IIIA

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Appendix

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Figure	Panel	Comparison	<i>P</i> -value
1	В	Tnfa; WT vs. MPSIIIA	<i>P</i> <0.0001
1	В	Il1b; WT vs. MPSIIIA	P = 0.0004
1	В	Il1a; WT vs. MPSIIIA	P = 0.0168
1	В	Il6; WT vs. MPSIIIA	P = 0.0089
1	В	Il1rn; WT vs. MPSIIIA	P = 0.0002

Appendix Table S1. List of exact P-values for graphs in Figure 1.

Appendix Table S2. List of exact P-values for graphs in Figure 2.

Figure	Panel	Comparison	<i>P</i> -value
2	В	Within 2 hours; Saline vs. MPSIIIA GAG	<i>P</i> < 0.0001
2	В	Within 6 hours; Saline vs. MPSIIIA GAG	<i>P</i> < 0.0001
2	В	Within MPSIIIA GAG; 1 hour vs. 2 hours	<i>P</i> < 0.0001
2	В	Within MPSIIIA GAG; 1 hour vs. 6 hours	P = 0.0178
2	В	Within MPSIIIA GAG; 2 hours vs. 6 hours	P = 0.0096
2	В	Within 1 hour; Saline vs. LPS	<i>P</i> < 0.0001
2	В	Within 2 hours; Saline vs. LPS	<i>P</i> < 0.0001
2	В	Within 6 hours; Saline vs. LPS	<i>P</i> < 0.0001
2	D	PBS vs. MPSIIIA GAG	<i>P</i> < 0.0001
2	D	PBS vs. LPS	<i>P</i> < 0.0001
2	D	WT GAG vs. MPSIIIA GAG	<i>P</i> < 0.0001
2	Е	PBS vs. MPSIIIA	P = 0.0033
2	Е	PBS vs. LPS	<i>P</i> < 0.0001
2	F	Vehicle vs. MPSIIIA GAG+vehicle	<i>P</i> <0.0001
2	F	MPSIIIA GAG+vehicle vs. MPSIIIA GAG+CLI-095	<i>P</i> <0.0001
2	G	MPSIIIA GAG vs. MPSIIIA GAG+cABC	P = 0.0007
2	G	MPSIIIA GAG vs. MPSIIIA GAG+HepM	<i>P</i> < 0.0001
2	G	MPSIIIA GAG vs. MPSIIIA GAG+HepM+cABC	<i>P</i> < 0.0001
2	G	MPSIIIA GAG+cABC vs. MPSIIIA GAG+HepM	<i>P</i> <0.0001
2	G	MPSIIIA GAG+cABC vs. MPSIIIA GAG+HepM+cABC	<i>P</i> < 0.0001
2	G	MPSIIIA GAG+HepM vs. MPSIIIA GAG+HepM+cABC	<i>P</i> = 0.0164

Figure	Panel	Comparison	<i>P</i> -value
3	С	Pycard; WT vs. MPSIIIA	P = 0.0241
3	С	Nlrp3; WT vs. MPSIIIA	P = 0.0283
3	С	Casp1; WT vs. MPSIIIA	P = 0.0131
3	С	Ctsb; WT vs. MPSIIIA	P = 0.0416
3	С	Casp4; WT vs. MPSIIIA	<i>P</i> = 0.0438
3	С	Gsdmd; WT vs. MPSIIIA	P = 0.0320
3	Е	Within MPSIIIA GAG; Vehicle vs. ATP	P = 0.0002
3	Е	Within LPS; Vehicle vs. ATP	<i>P</i> = 0.0492
3	F	Within MPSIIIA GAG; Vehicle vs. CHC	P = 0.0001
3	F	Within LPS; Vehicle vs. CHC	P = 0.0027
3	G	Within MPSIIIA GAG; Vehicle vs. rhAß	P = 0.0052
3	G	Within LPS; Vehicle vs. rhAß	P = 0.0062
3	Н	Vehicle alone vs. LPS+vehicle	P = 0.0006
3	Н	Vehicle alone vs. LPS+ATP	P = 0.0001
3	Ι	Vehicle alone vs. LPS+vehicle	<i>P</i> < 0.0001
3	Ι	Vehicle alone vs. LPS+CHC	<i>P</i> < 0.0001
3	J	Vehicle alone vs. LPS+vehicle	<i>P</i> < 0.0001
3	J	Vehicle alone vs. LPS+ rhAβ	<i>P</i> < 0.0001

Appendix Table S3. List of exact P-values for graphs in Figure 3.

Appendix Table S4. List of exact P-values for graphs in Figure 4.

Figure	Panel	Comparison	<i>P</i> -value
4	В	Within NTC; no treatment vs. 10 ng/ml IL-1β	<i>P</i> <0.0001
4	В	Within LV.GFP; no treatment vs. 10 ng/ml IL-1β	<i>P</i> <0.0001
4	В	Within 10 ng/ml IL-1β; NTC vs. LV.IL1RN	<i>P</i> <0.0001
4	В	Within 10 ng/ml IL-1β; LV.GFP vs. LV.IL1RN	<i>P</i> <0.0001

Appendix Table S5. List of exact P-values for graphs in Figure 5.

Figure	Panel	Comparison	<i>P</i> -value
5	С	WT vs. MPSIIIA	<i>P</i> = 0.0348
5	С	MPSIIIA vs. MPSIIIA+LV.IL1RN	P = 0.0062
5	С	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	P = 0.0278
5	Е	WT vs. MPSIIIA	<i>P</i> < 0.0001
5	Е	MPSIIIA vs. MPSIIIA+LV.IL1RN	P = 0.0002
5	Е	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001

Figure	Panel	Comparison	<i>P</i> -value
6	С	WT vs. MPSIIIA	<i>P</i> < 0.0001
6	С	WT vs. MPSIIIA+LV.IL1RN	P = 0.0023
6	С	WT vs. MPSIIIAxIL-1R1 ^{-/-}	P = 0.0040
6	С	MPSIIIA vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	С	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	С	WTxIL-1R1 ^{-/-} vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	D	WT vs. MPSIIIA	<i>P</i> < 0.0001
6	D	WT vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	D	WT vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	D	MPSIIIA vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	D	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	D	WTxIL-1R1 ^{-/-} vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	Е	WT vs. MPSIIIA	<i>P</i> < 0.0001
6	Е	WT vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	Е	WT vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	Е	MPSIIIA vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	Е	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	Е	WTxIL-1R1 ^{-/-} vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	F	WT vs. MPSIIIA	<i>P</i> < 0.0001
6	F	WT vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	F	WT vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	F	MPSIIIA vs. MPSIIIA+LV.IL1RN	<i>P</i> < 0.0001
6	F	MPSIIIA vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001
6	F	MPSIIIA+LV.IL1RN vs. MPSIIIAxIL-1R1-/-	<i>P</i> < 0.0001
6	F	WTxIL-1R1 ^{-/-} vs. MPSIIIAxIL-1R1 ^{-/-}	<i>P</i> < 0.0001

Appendix Table S6. List of exact P-values for graphs in Figure 6.

Appendix Table S7. List of exact P-values for graphs in Expanded View Figures.

Figure	Panel	Comparison	<i>P</i> -value
EV1	В	UA2S-GlcNS6S; WT vs. MPSIIIA	<i>P</i> < 0.0001
EV1	В	UA-GlcNS6S; WT vs. MPSIIIA	P = 0.0044
EV1	В	UA2S-GlcNS; WT vs. MPSIIIA	<i>P</i> < 0.0001
EV1	В	UA-GlcNS; WT vs. MPSIIIA	<i>P</i> < 0.0001
EV1	В	UA-GlcNAc6S; WT vs. MPSIIIA	<i>P</i> < 0.0001
EV1	В	UA-GlcNAc; WT vs. MPSIIIA	<i>P</i> < 0.0001
EV2		MPSIIIA GAG vs. MPSIIIA GAG+CHC	<i>P</i> < 0.0001
EV2		MPSIIIA GAG+CHC vs. MPSIIIA GAG+CHC+VX-765	P = 0.0003
EV2		MPSIIIA GAG+CHC vs. MPSIIIA GAG+CHC+CA-074Me	P = 0.0001
EV2		MPSIIIA GAG+CHC vs. MPSIIIA GAG+CHC+CytD	P = 0.0004
EV2		MPSIIIA GAG+CHC vs. MPSIIIA GAG+CHC+KCl	P = 0.0003
EV3	В	WT vs. MPSIIIA	P = 0.0097
EV3	В	WT vs. WTxIL-1R1 ^{-/-}	P = 0.0314
EV3	В	MPSIIIA vs. MPSIIIA+LV.IL1RN	P = 0.0071
EV3	В	MPSIIIA+LV.IL1RN vs. MPSIIIAxIL-1R1 ^{-/-}	P = 0.0158
EV4	В	WT vs. MPSIIIA	P = 0.0003
EV4	В	WT vs. MPSIIIA+LV.IL1RN	P = 0.0078
EV4	В	WT vs. MPSIIIAxIL-1R1 ^{-/-}	P = 0.0348
EV4	В	WTxIL-1R1 ^{-/-} vs. MPSIIIAxIL-1R1 ^{-/-}	P = 0.0008