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

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

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

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

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

Figure	Panel	Comparison	P-value
3	C	Pycard; WT vs. MPSIIIA	$P = 0.0241$
3	C	Nlrp3; WT vs. MPSIIIA	$P = 0.0283$
3	C	Casp1; WT vs. MPSIIIA	$P = 0.0131$
3	C	Ctsb; WT vs. MPSIIIA	$P = 0.0416$
3	C	Casp4; WT vs. MPSIIIA	$P = 0.0438$
3	C	Gsdmd; WT vs. MPSIIIA	$P = 0.0320$
3	E	Within MPSIIIA GAG; Vehicle vs. ATP	$P = 0.0002$
3	E	Within LPS; Vehicle vs. ATP	$P = 0.0492$
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. rhA β	$P = 0.0052$
3	G	Within LPS; Vehicle vs. rhA β	$P = 0.0062$
3	H	Vehicle alone vs. LPS+vehicle	$P = 0.0006$
3	H	Vehicle alone vs. LPS+ATP	$P = 0.0001$
3	I	Vehicle alone vs. LPS+vehicle	$P < 0.0001$
3	I	Vehicle alone vs. LPS+CHC	$P < 0.0001$
3	J	Vehicle alone vs. LPS+vehicle	$P < 0.0001$
3	J	Vehicle alone vs. LPS+ rhA β	$P < 0.0001$

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

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

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

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

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

Figure	Panel	Comparison	P-value
6	C	WT vs. MPSIII A	$P < 0.0001$
6	C	WT vs. MPSIII A+LV.IL1RN	$P = 0.0023$
6	C	WT vs. MPSIII AxIL-1R1 ^{-/-}	$P = 0.0040$
6	C	MPSIII A vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	C	MPSIII A vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	C	WTxIL-1R1 ^{-/-} vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	D	WT vs. MPSIII A	$P < 0.0001$
6	D	WT vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	D	WT vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	D	MPSIII A vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	D	MPSIII A vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	D	WTxIL-1R1 ^{-/-} vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	E	WT vs. MPSIII A	$P < 0.0001$
6	E	WT vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	E	WT vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	E	MPSIII A vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	E	MPSIII A vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	E	WTxIL-1R1 ^{-/-} vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	F	WT vs. MPSIII A	$P < 0.0001$
6	F	WT vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	F	WT vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	F	MPSIII A vs. MPSIII A+LV.IL1RN	$P < 0.0001$
6	F	MPSIII A vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	F	MPSIII A+LV.IL1RN vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$
6	F	WTxIL-1R1 ^{-/-} vs. MPSIII AxIL-1R1 ^{-/-}	$P < 0.0001$

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

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