

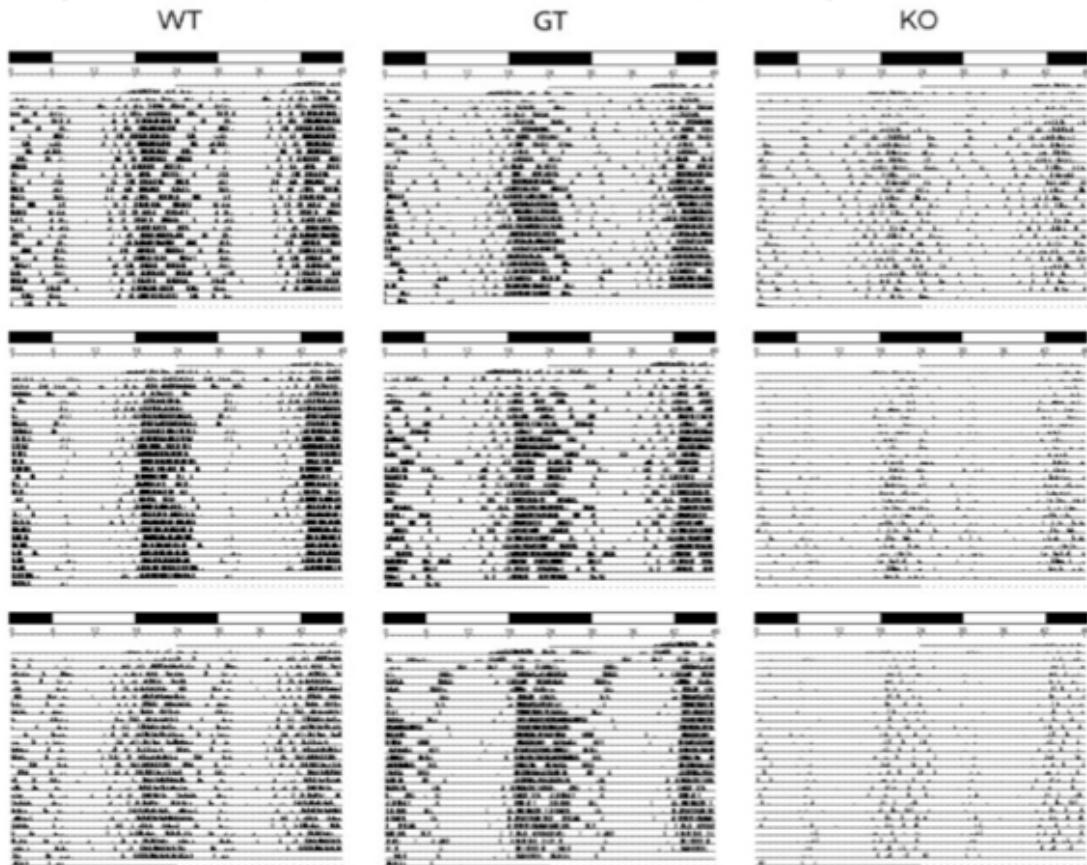
Supplemental Information

Lentiviral Hematopoietic Stem Cell Gene

Therapy Corrects Murine Pompe Disease

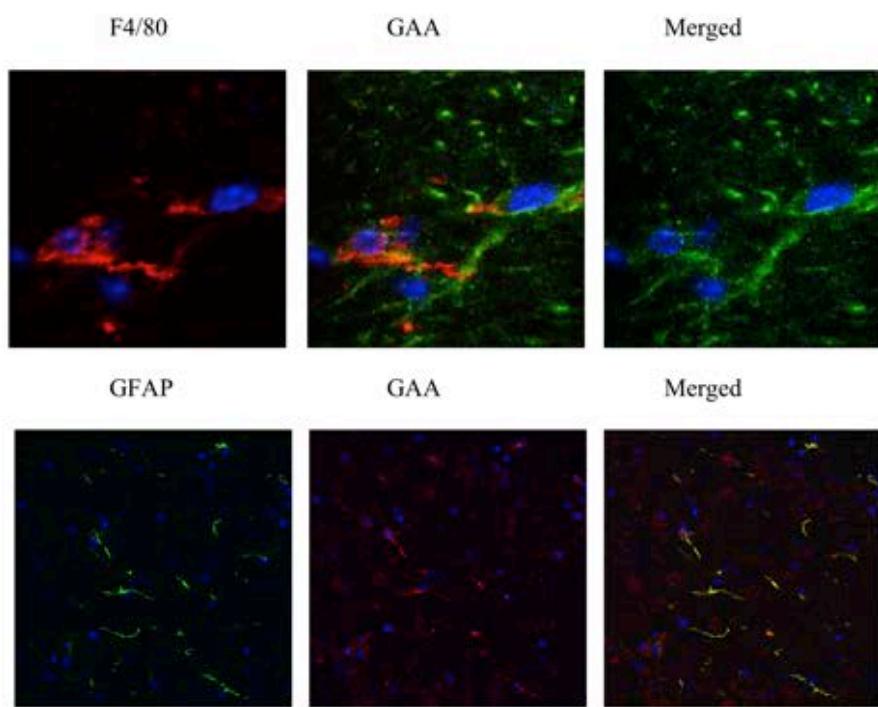
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S1 Fig. Circadian rhythm is maintained in voluntary running wheel exercises.



The actograms represent circadian locomotor activity and rest cycles. The interrupted horizontal bar above the actograms displays day (white) and night (black) cycles. A 48-hour time frame is shown depicting running periods (in black) and resting periods out of the running wheels. Three representative mice are displayed for each condition, i.e. wild-type (WT), GAAco treated *Gaa*^{-/-} mice (GT) and *Gaa*^{-/-} mice (KO). All animals became more active during the night and all groups showed similar day/night activity patterns, except that KO mice were considerably less active.

S2 Fig. GAA in microglia (upper panel, magnification x 100) and astrocytes (magnification x 10).



Representative areas of microglia with in green the GAA staining and in red the F4/80 staining, whereas blue represents the Hoechst staining. In the astrocytes, red represents GAA staining and green the astrocytes GFAP staining, showing that virtually all astrocytes stain positive for GAA.

S1 Table. Common integration sites

Vector	Num. of samples	Total IS	Unique Genes	IS within 10kb of TSS	IS within 10kb of gene	Intronic IS
GAAco	36	333	310	65	221	180
GFP	5	424	388	91	304	249
Continuation:						
Unique Oncogenes	Total oncogenes	CIS	CIS oncogenes	Frequency oncogenes	Frequency intronic IS	Frequency IS w/in 10kb of gene
12	14	15	2	4.2%	54.1%	66.4%
12	13	25	1	3.1%	58.7%	71.7%

S2 Table. List of all CIS, oncogenes in red

vector	CISorder	Chromosome	Gene	Loci
GAaco	2	1	Cr2	197025311;197025462
GAaco	2	8	Eps15l1	74919901;74919904
GAaco	2	X	Cybb	9041400;9041771
GAaco	2	1	Smap1	23885960;23885961
GAaco	2	1	37500	95387308;95387315
GAaco	2	1	Mcm6	130277570;130277585
GAaco	2	1	Scama3a	161234959;161234977
GAaco	2	1	Gm4846	168411650;168411781
GAaco	2	1	Cr1l	198946840;198946841
GAaco	2	9	Trpc6	8581308;8581617
GAaco	2	9	Elov15	77797454;77797462
GAaco	2	10	Mir1929	44061578;44061740
GAaco	2	10	Ascc3	50315191;50315792
GAaco	2	16	Mir28	24902396;24902457
GAaco	2	18	Tmx3	90893725;90712409
GFP	2	18	Zfp521	13872964;13873209
GFP	2	2	Plxdc2	16282665;16282854
GFP	2	3	Gyg	20021989;20022410
GFP	2	3	Hist2h2aa2	98047653;98048217
GFP	2	3	Sfrs11	157683042;157707831
GFP	2	5	Pcdh7	58178866;58187690
GFP	2	5	Cenpc1	86387408;86387768
GFP	2	5	AU042671	121751014;121751172
GFP	2	6	Cul1	47445391;47464266
GFP	2	6	Bicd1	149497766;149508206
GFP	2	8	Large	75821352;75821490
GFP	2	9	Slc37a2	37102871;37103208
GFP	2	10	Jmjdc1c	66660521;66689907
GFP	2	11	Sf1	3086225;3095580
GFP	2	11	Slfn1	82909694;82925619
GFP	2	11	H3f3b	115887991;115888197
GFP	2	12	Stxbp6	46220785;46221361
GFP	2	13	Hrh2	54307859;54308041
GFP	2	13	Edil3	89376126;89376754
GFP	2	15	Abra	41616255;41616522
GFP	2	15	Taf2	54871080;54871277
GFP	2	15	Phf20l1	66402373;66402700
GFP	2	16	Cggbp1	64821368;64821516
GFP	2	16	Gbe1	70413892;70418655
GFP	2	18	Wac	7873989;7901856