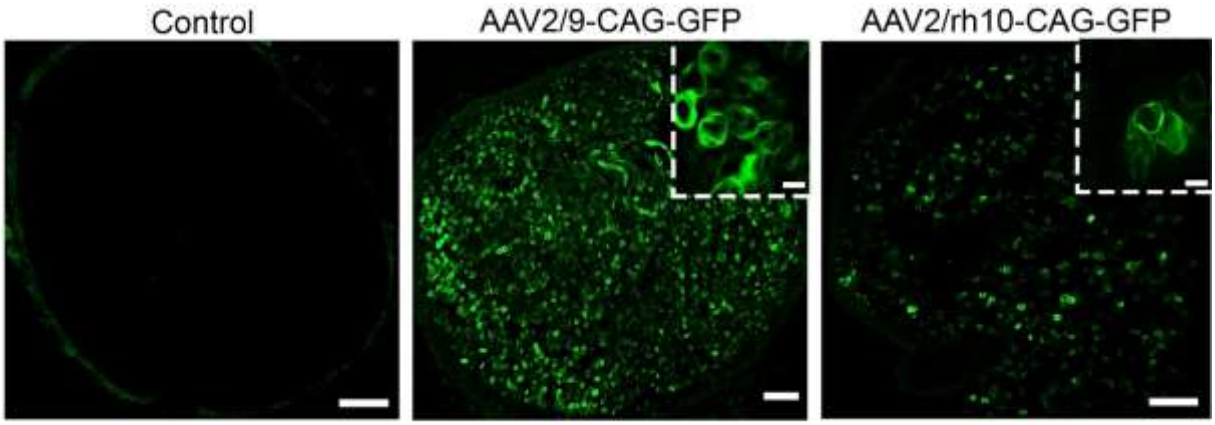

Fig. S1 AAV2/9 and AAV2/rh10-CAG-GFP transduce mSC after intra-nerve injection in mouse. Representative images of sciatic nerve cross-sections showing GFP protein expression at the injection site after intra-nerve injection of AAV2/9 and AAV2/rh10-CAG-GFP in adult mouse (2-3 months old, 5 x 10^{10} vg/nerve in 8 µl, *n* = 3 animals per group). Control represents an adult mouse sciatic nerve injected with Fast Green alone. All animals were sacrificed one month post-injection. Inserts show the circular shape of transduced cells. Scale bars: 100 µm and 10 µm for the inserts.

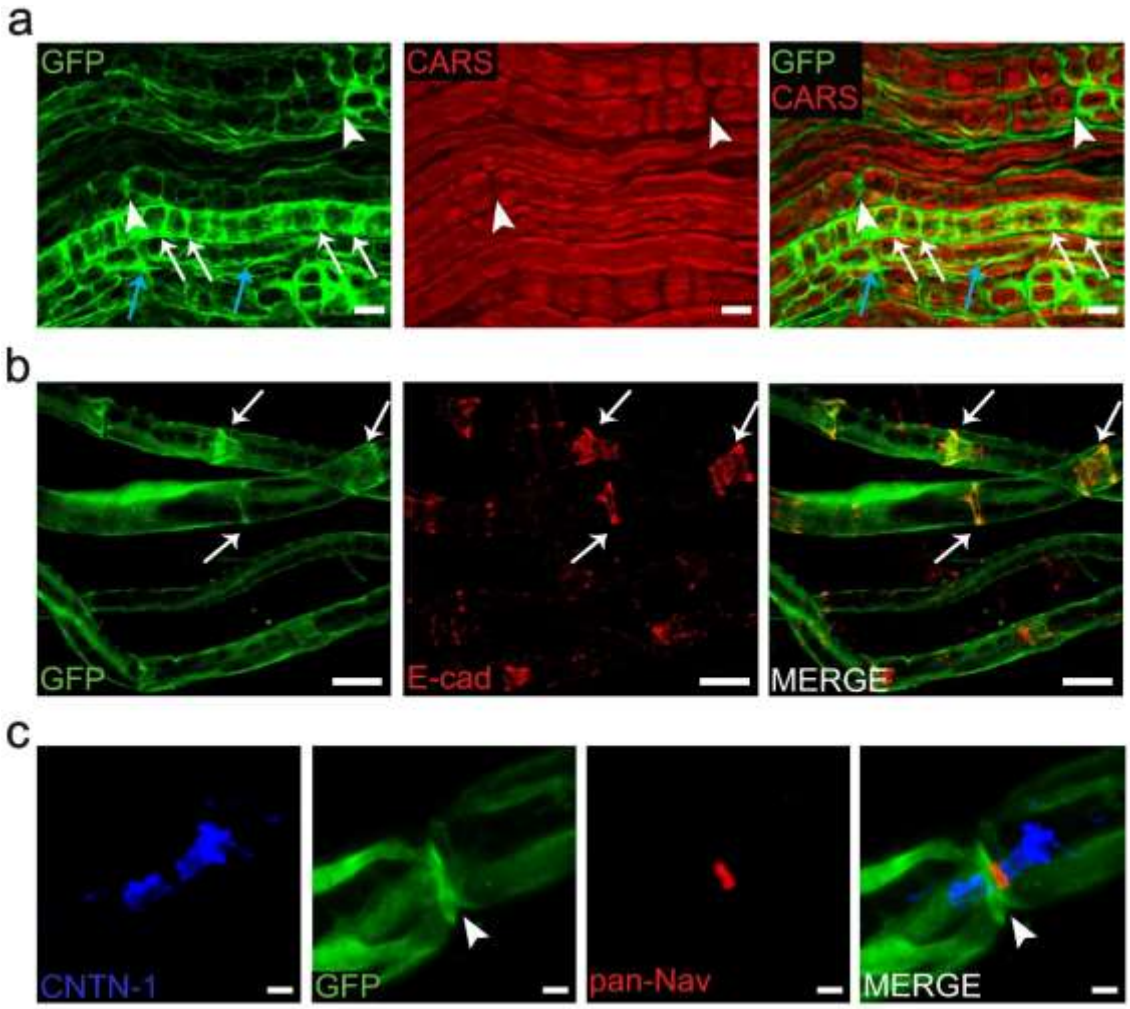

Fig. S2 GFP is expressed in mSC after intra-nerve injection. **a** GFP (green) and CARS (red) imaging of sciatic nerve injected with AAV2/9 expressing GFP in rat pup (1 x 10^{11} vg/nerve in 8 µl, *n* = 3 animals) twelve months post-injection showing specific features of transduced mSC, such as Schmidt-Lanterman incisures (white arrows), Cajal's band (blue arrows) and paranodal loops surrounding nodes of Ranvier (arrrowheads). Scale bar: 10 µm. **b** and **c** Representative images of immunostainings on teased fibers from a rat sciatic nerve injected with AAV2/9-CAG-GFP (injection at P6-P7, 1 x 10^{11} vg/nerve in 8 µl, *n* = 3 animals) and sacrificed one month post-injection. GFP protein is expressed in Schmidt-Lanterman incisures immunostained with E-cadherin (E-cad, red) (**b**) and paranodal loops surrounding nodes of Ranvier immunostained with pan-Nav (red) and paranodes immunostained with Contactin-1 (CNTN-1, blue) (**c**). Scale bars: 10 µm (**b**) and 2 µm (**c**).

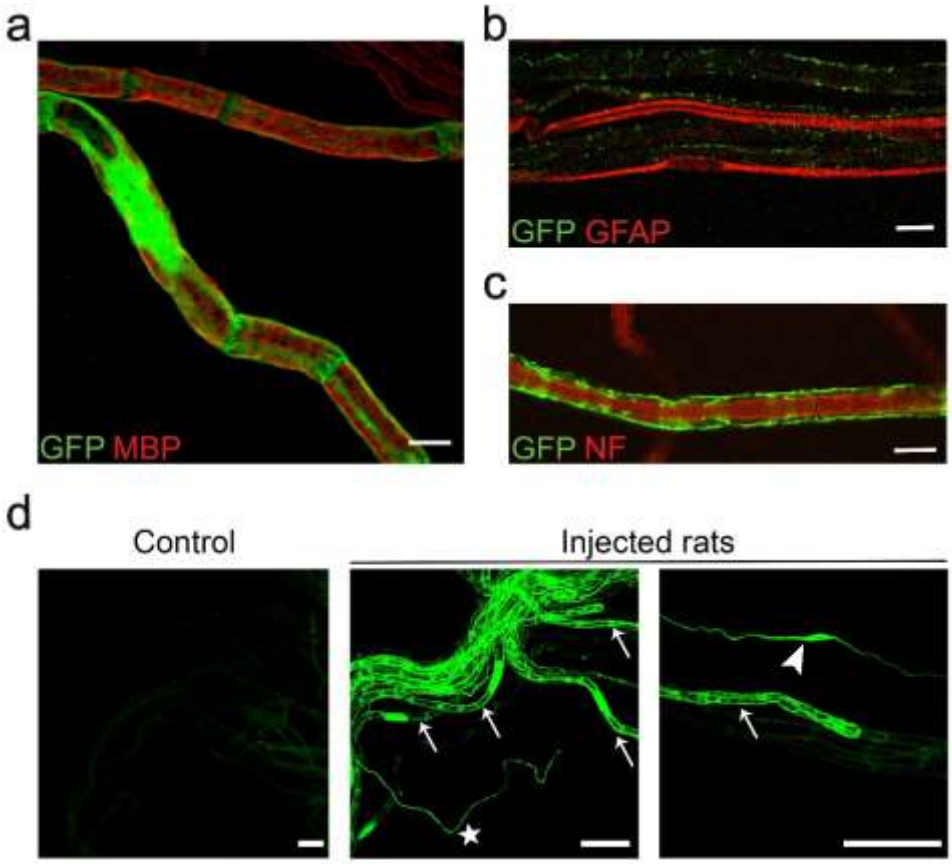

Fig. S3 AAV2/9-CAG-GFP transduces more specifically mSC than other cells after intra-nerve injection. Representative images of teased fibers from a rat sciatic nerve injected with AAV2/9-CAG-GFP (injection at P6-P7, 1 x 10^{11} vg/nerve in 8 µl, *n* = 3 animals) and sacrificed one month post-injection. Immunostainings with MBP (**a**), GFAP (**b**) and Neurofilament (NF) (**c**) as markers for mSC, nmc and axon respectively show that GFP labels mSC. Scale bars: 10 µm. **d** In addition to mSC (arrows), GFP labels some nmc (arrowhead) and axons (stars). Control represents a rat pup sciatic nerve injected with Fast Green alone. Scale bars: 50 µm.

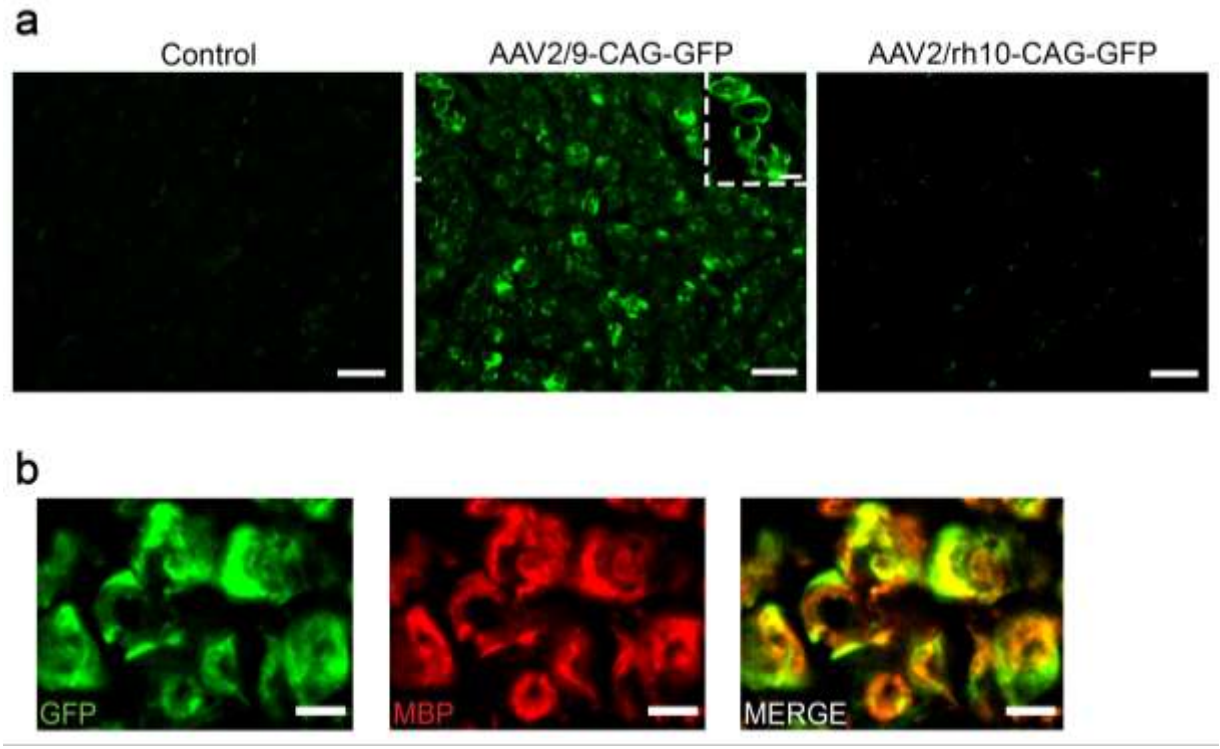

Fig. S4 AAV2/9-CAG-GFP transduces mSC more efficiently than AAV2/rh10-CAG-GFP after intra-nerve injection in NHP sciatic nerves. a Representative images of sciatic nerve cross-sections showing GFP protein expression at the injection site after intra-nerve injection of AAV2/9 and AAV2/rh10-CAG-GFP in adult NHP (3.7 and 2.3 years old for AAV2/9 and AAV2/rh10, 5 x 10^{12} vg/nerve in 416 µl, *n* = 1 animal per group). Control shows the contralateral sciatic nerve. All animals were sacrificed one month post-injection. Inserts show the circular shape of transduced mSC. Scale bars: 50 µm and 10 µm for the inserts. **b** Representative images of immunostaining on sciatic nerve cross-sections of adult NHP at the injection site using myelin MBP marker (red) (*n* = 3 nerve cross-sections per NHP). GFP (green) partially colocalizes with MBP. Scale bar: 10 µm.

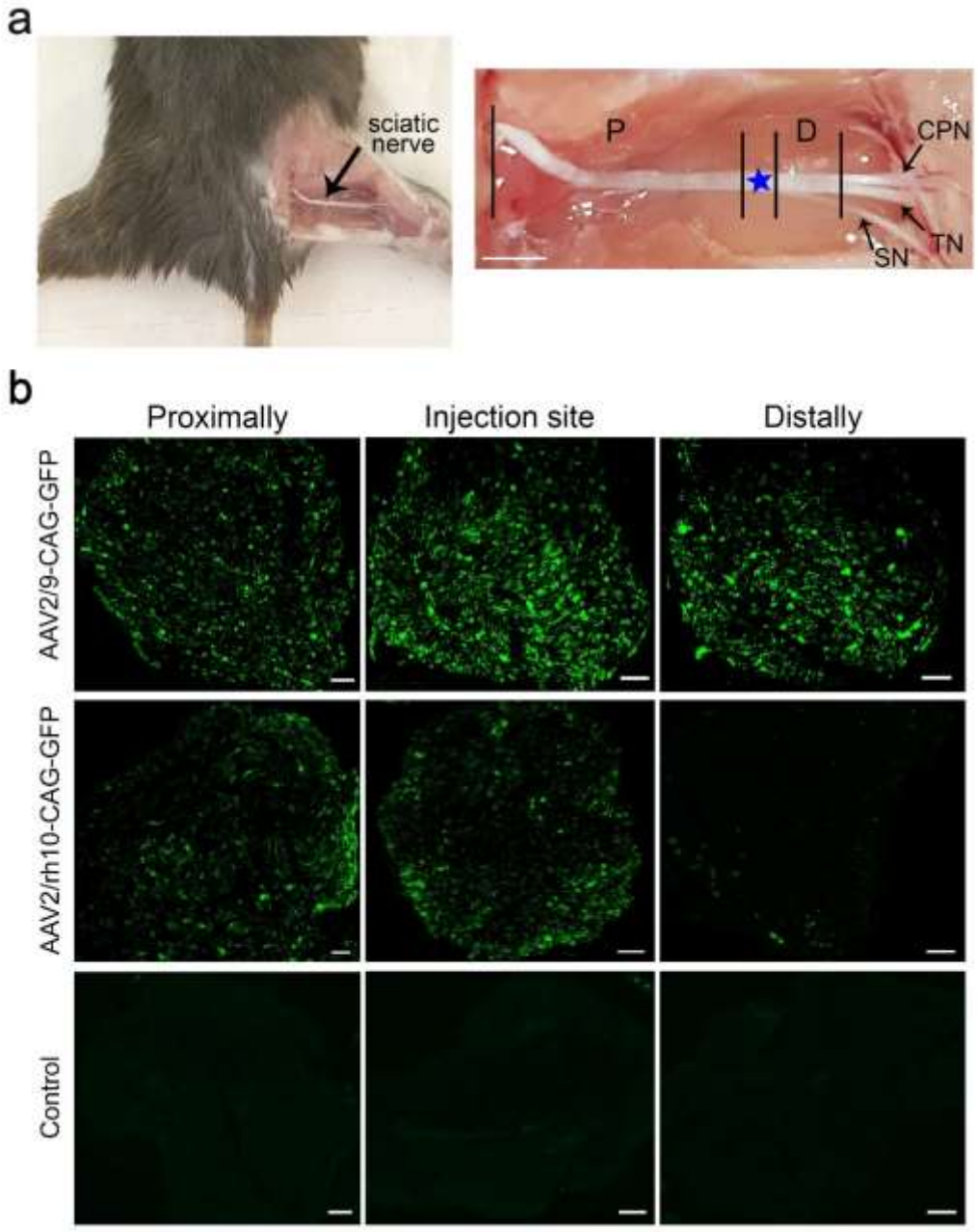

Fig. S5 AAV2/9-CAG-GFP exhibits a higher diffusion along the sciatic nerve than AAV2/rh10-CAG-GFP mSC after intra-nerve injection in rodents. a Left: Dorsal view of a mouse showing the localization of the right sciatic nerve. **Right**: Higher magnification of the right sciatic nerve showing the injection site (blue star) and the proximal (P) and the distal (D) parts. CPN: Common peroneal nerve, TN: tibial nerve, SN: sural nerve. Scale bar: 0.5mm. **b** Representative images of sciatic nerve cross-sections showing GFP protein expression at the injection site, proximally and distally, after intra-nerve injection of AAV2/9 and AAV2/rh10-CAG-GFP in rat pup (P6-P7, 1 x 10^{11} vg/nerve in 8 µl, *n* = 3 animals per group). Control represents a rat pup sciatic nerve injected with Fast Green alone. All animals were sacrificed one month post-injection. Scale bars: 50 µm.

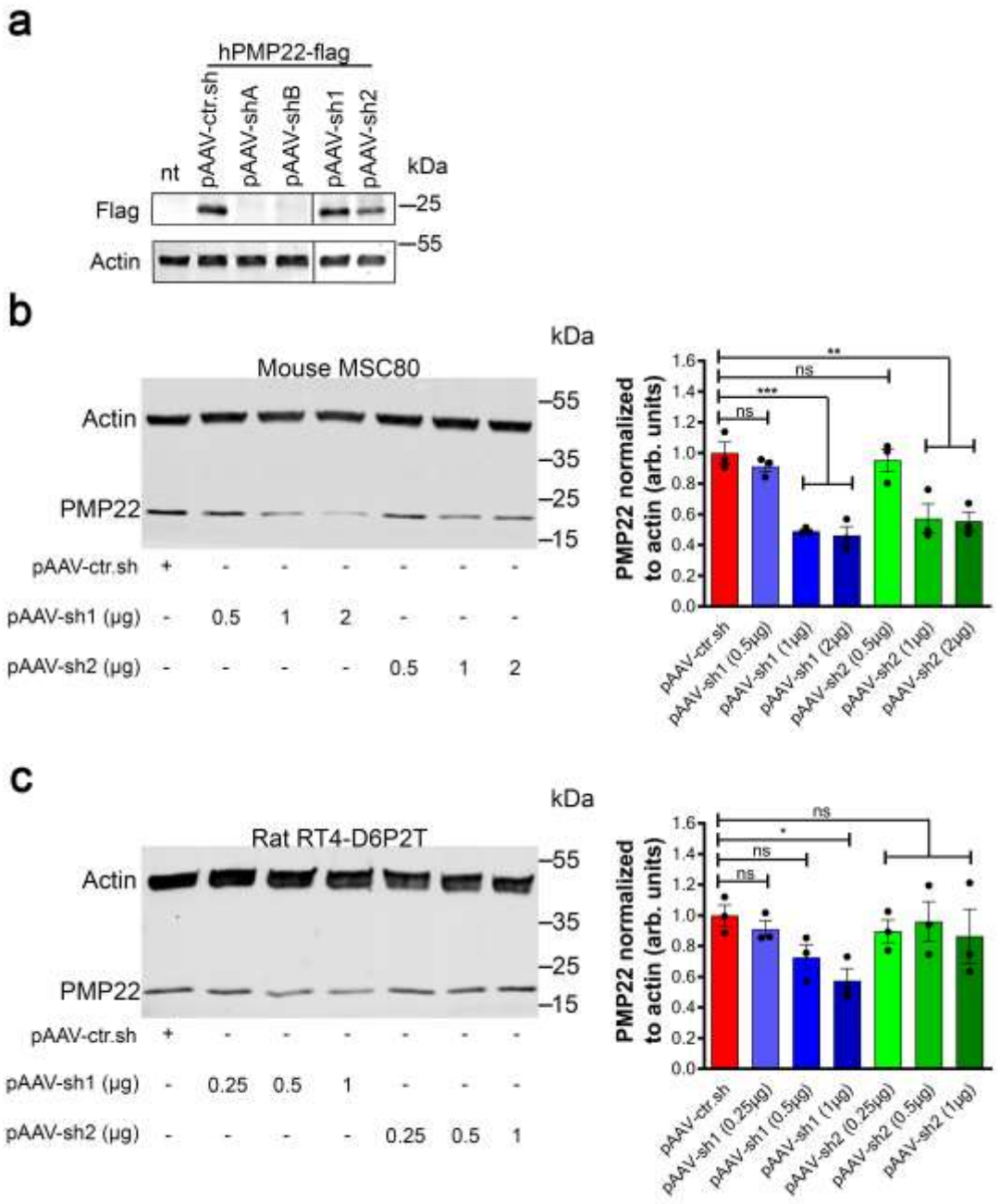

Fig. S6 ***In vitro*** **validation of shRNAs targeting human or rodent PMP22 expression. a** Representative Western blot of three independent experiments showing human Flag-tagged PMP22 detected with Flag antibody (Flag) and β-actin as loading control (Actin). HEK293 cells were non-transfected (nt) or cotransfected with pCMV3 human Flag-tagged PMP22 plasmid (hPMP2-Flag) and pAAV-ctr.sh, pAAV-shA, pAAV-shB (both targeting human *PMP22* mRNA), pAAV-sh1 or pAAV-sh2 (both targeting mouse *Pmp22* mRNA). All bands were obtained on the same membrane but not contiguously (black line separates the two parts). **b Left**: Western blot analysis showing mouse PMP22 protein expression in mouse MSC80 Schwann cell line transfected with pAAV-sh1, pAAV-sh2 or pAAV-ctr.sh at the indicated dose per well (µg of DNA). Actin expression is used as loading control. **Right**: quantitation of mouse PMP22 expression normalized to actin (n = 3 independent experiments). Statistical tests

show one-way ANOVA followed by Dunnett's post hoc test, two-sided. *** $p = 0.0003$ between pAAV-ctr.sh and pAAV-sh1 (1 µg), *** $p = 0.0002$ between pAAV-ctr.sh and pAAV-sh1 (2 µg), ** $p = 0.0014$ between pAAV-ctr.sh and pAAV-sh2 (1 µg) and *** $p = 0.001$ between pAAV-ctr.sh and pAAV-sh2 (2 µg); ns, not significant; arb. units, arbitrary unit. All error bars show SEM **c Left**: Western blot analysis of rat PMP22 protein expression in rat RT4-D6P2T Schwann cell line transfected with pAAV-sh1, pAAV-sh2 or pAAV-ctr.sh at the indicated dose per well (µg of DNA). Actin expression is used as loading control**. Right**: quantitation of mouse PMP22 expression normalized to actin ($n = 3$ experiments independent). Statistical tests show one-way ANOVA followed by Dunnett's post hoc test, two-sided. * $p = 0.0495$ between pAAV-ctr.sh and pAAV-sh1 (1 µg); ns, not significant; arb. units, arbitrary unit. All error bars show SEM. Source data are provided as a Source Data file.

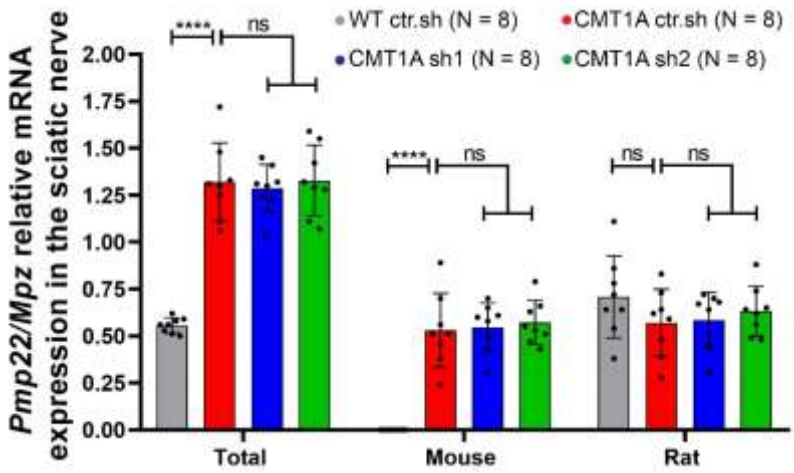

Fig. S7 Intra-nerve treatments with AAV2/9-sh1 and -sh2 do not affect *Pmp22* mRNA expression in rat CMT1A sciatic nerves. Mean level of total *Pmp22*, mouse *Pmp22* and rat *Pmp22* mRNA normalized on rat *Mpz* mRNA level in sciatic nerve of WT ctr.sh, CMT1A ctr.sh, CMT1A sh1 or CMT1A sh2 three months after injection. (n = 8 animals per group). *ActB* and *Rsp9* mRNA levels were used as reference genes. Statistical test shows one-way ANOVA followed by Dunnett's post hoc test, two-sided. For total *Pmp22*/*Mpz* relative mRNA expression, **** $p < 0.0001$ between WT ctr.sh and CMT1 ctr.sh; for mouse *Pmp22*/*Mpz* relative mRNA expression, **** $p < 0.0001$ between WT ctr.sh and CMT1 ctr.sh; ns, not significant. All error bars show SD. Source data are provided as a Source Data file.

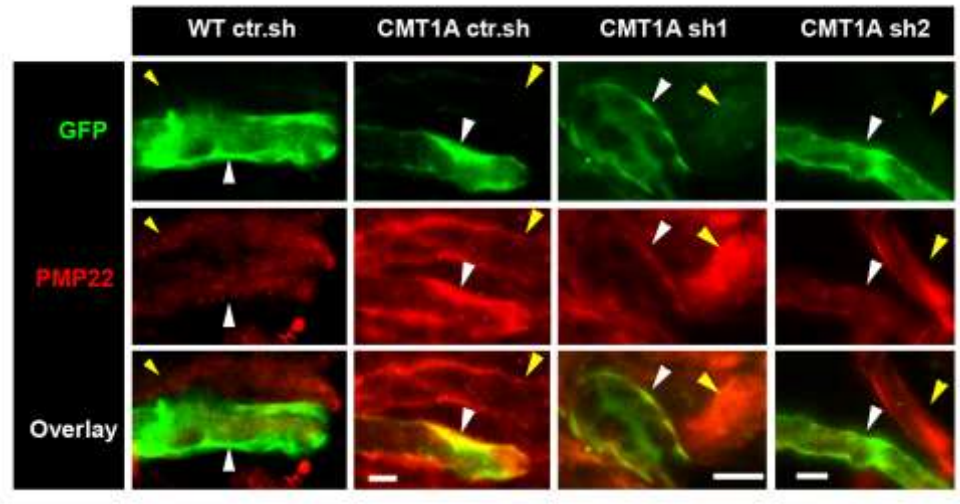

Fig. S8 Intra-nerve injections of AAV2/9-sh1 and sh2 decrease PMP22 expression in CMT1A rat mSC

Representative images (*n* = 3 animals per group) obtained from cryo-sections of WT ctr.sh, CMT1A ctr.sh, CMT1A sh1 and CMT1A sh2 sciatic nerves were stained for PMP22 (red) and imaged using a confocal microscope. White arrowheads show transduced mSC expressing GFP (green) and yellow arrowheads non-transduced mSC. In WT ctr.sh and CMT1A ctr.sh nerves transduced cells show a similar amount of PMP22 as non-transduced cells. In CMT1A sh1 and CMT1A sh2 nerves transduced cells show less PMP22 than non-transduced cells, showing that AAV2/9-sh1 and -sh2 decrease PMP22 expression in CMT1A rat mSC. Animals were sacrificed three months post-injection. Scale bars= 5 µm.

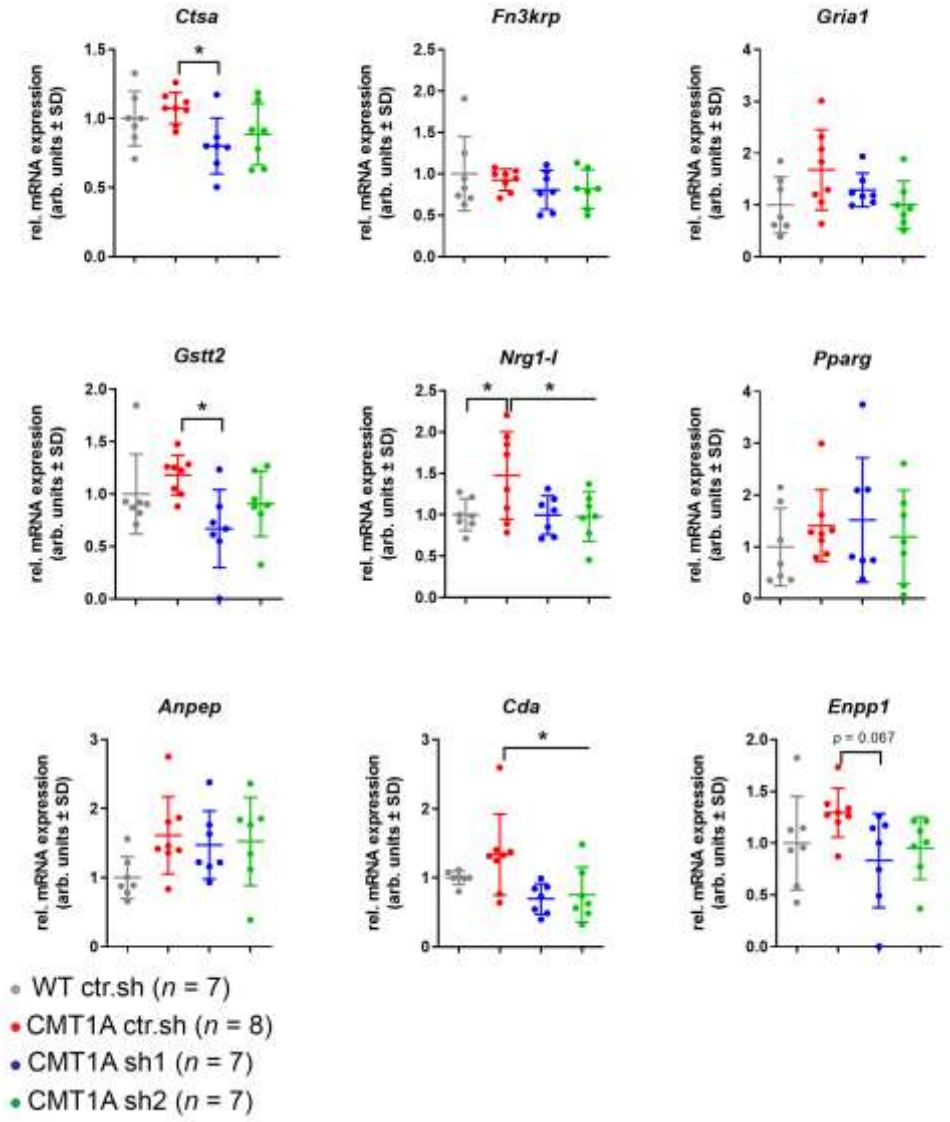

Fig. S9 Transcriptomic analysis of nine biomarkers in the front paw skin. The mRNA expressions of nine biomarkers (label on the top of each graph) were quantified using RT-qPCR in the skin of front paws (n = 7 animals for WT ctr.sh, n = 8 animals for CMT1A ctr.sh, n = 7 animals for CMT1A and n = 7 animals for CMT1A sh2) and normalized against the mRNA expression levels of the stable housekeeping gene *ActB*. Relative (rel.) mRNA expressions are are expressed as the mean in arbitrary unit (arb. units). Error bars show SD. Statistical tests show one-way ANOVA followed by Sidack's post hoc test, two-sided. For *Ctsa*, * p = 0.0262 between CMT1A ctr.sh and CMT1A sh1; for *Gstt2*, * p = 0.0139 between CMT1A ctr.sh and CMT1A sh1; for *Nrg1-I*, * p = 0.0429 between WT ctr.sh and CMT1A ctr.sh , * p = 0.0409 between CMT1A ctr.sh and CMT1A sh1, * p = 0.0324 between CMT1A ctr.sh and CMT1A sh2; for *Cda*, * p = 0.0107 between CMT1A ctr.sh and CMT1A sh1, * p = 0.0226 between CMT1A ctr.sh and CMT1A sh2. When statistical test results are not shown, this indicates non-significant results. Source data are provided as a Source Data file.

Supplementary Table S1

		Transduction rate in adult NHP (%)	
		AAV2/9-CAG-GFP	AAV2/rh10-CAG-GFP
Proximally	4 cm from injection site	21 ± 2	< 1
	2 cm from injection site	55 ± 4	< 1
Injection site		68 ± 3	< 1
Distally	2 cm from injection site	69 ± 5	< 1

Quantification of the transduction pattern after intra-nerve injections of AAV2/9 and AAV2/rh10-CAG-GFP in NHP. Adult NHP were injected at 3,7 and 2.3 years old with AAV2/9 and AAV2/rh10-CAG-GFP respectively. All animals were sacrificed one month post-injection. The transduction rate is the percentage of transduced mSC (GFP and MBP positive cells) on the overall number of mSC (MBP positive cells) per section. Proximally distances of the injection were 2 and 4 cm. Distal distance of the injection site was 2 cm. The results are expressed as the mean ± SD. Source data are provided as a Source Data file.

Supplementary Table S2 Multiple comparison tests for data presented in Fig. 5

NCV (m/s)

Within each row, compare columns (simple effects within rows)	
Number of families	6
Number of comparisons per family	6
Alpha	0,05

Tukey's multiple comparisons test, two-sided	Mean Diff,	95,00% CI of diff,	Significant?	Summary	Adjusted *p*-Value
1 month					
WT scr.-shRNA vs. CMT1A scr.-shRNA	23,23	19,58 to 26,88	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	3,244	-1,639 to 8,128	No	ns	0,2451
WT scr.-shRNA vs. CMT1A shRNA50	4,830	-0,7567 to 10,42	No	ns	0,0959
CMT1A scr.-shRNA vs. CMT1A shRNA49	-19,99	-24,68 to -15,29	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-18,40	-23,85 to -12,94	Yes	****	<0,0001
CMT1A shRNA49 vs. CMT1A shRNA50	1,586	-4,492 to 7,663	No	ns	0,8630
2 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	28,19	17,62 to 38,75	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	11,85	1,622 to 22,08	Yes	*	0,0256
WT scr.-shRNA vs. CMT1A shRNA50	14,30	3,763 to 24,84	Yes	**	0,0093
CMT1A scr.-shRNA vs. CMT1A shRNA49	-16,34	-22,35 to -10,32	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-13,88	-21,02 to -6,751	Yes	***	0,0004
CMT1A shRNA49 vs. CMT1A shRNA50	2,453	-3,479 to 8,386	No	ns	0,5912
3 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	32,34	20,29 to 44,38	Yes	***	0,0001
WT scr.-shRNA vs. CMT1A shRNA49	9,355	-3,443 to 22,15	No	ns	0,1818
WT scr.-shRNA vs. CMT1A shRNA50	13,38	0,3849 to 26,38	Yes	*	0,0431
CMT1A scr.-shRNA vs. CMT1A shRNA49	-22,98	-31,66 to -14,31	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-18,95	-28,10 to -9,811	Yes	***	0,0005
CMT1A shRNA49 vs. CMT1A shRNA50	4,028	-6,474 to 14,53	No	ns	0,6736
6 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	27,37	12,51 to 42,23	Yes	***	0,0007

WT scr.-shRNA vs. CMT1A shRNA49	2,244	-14,20 to 18,69	No	ns	0,9764
WT scr.-shRNA vs. CMT1A shRNA50	0,6541	-12,28 to 13,59	No	ns	0,9984
CMT1A scr.-shRNA vs. CMT1A shRNA49	-25,12	-40,89 to -9,365	Yes	**	0,0025
CMT1A scr.-shRNA vs. CMT1A shRNA50	-26,72	-38,36 to -15,07	Yes	***	0,0003
CMT1A shRNA49 vs. CMT1A shRNA50	-1,590	-15,75 to 12,57	No	ns	0,9826
9 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	37,74	29,36 to 46,11	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	7,325	-1,824 to 16,47	No	ns	0,1336
WT scr.-shRNA vs. CMT1A shRNA50	8,461	-1,136 to 18,06	No	ns	0,0899
CMT1A scr.-shRNA vs. CMT1A shRNA49	-30,41	-39,86 to -20,97	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-29,27	-39,14 to -19,41	Yes	****	<0,0001
CMT1A shRNA49 vs. CMT1A shRNA50	1,136	-9,290 to 11,56	No	ns	0,9877
12 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	36,01	24,62 to 47,41	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	11,11	-1,065 to 23,29	No	ns	0,0778
WT scr.-shRNA vs. CMT1A shRNA50	11,48	-4,036 to 26,99	No	ns	0,1761
CMT1A scr.-shRNA vs. CMT1A shRNA49	-24,90	-35,27 to -14,53	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-24,53	-39,12 to -9,942	Yes	**	0,0024
CMT1A shRNA49 vs. CMT1A shRNA50	0,3673	-14,67 to 15,40	No	ns	0,9998

Rotarod, Latency to fall (s)

Within each row, compare columns (simple effects within rows)	
Number of families	6
Number of comparisons per family	6
Alpha	0,05

Tukey's multiple comparisons test, two-sided	Mean Diff,	95,00% CI of diff,	Significant?	Summary	Adjusted *p*-Value

1 month					
WT scr.-shRNA vs. CMT1A scr.-shRNA	28,14	9,336 to 46,95	Yes	**	0,0044
WT scr.-shRNA vs. CMT1A shRNA49	16,10	-1,569 to 33,76	No	ns	0,0779
WT scr.-shRNA vs. CMT1A shRNA50	25,57	8,638 to 42,50	Yes	**	0,0038
CMT1A scr.-shRNA vs. CMT1A shRNA49	-12,05	-32,66 to 8,561	No	ns	0,3481
CMT1A scr.-shRNA vs. CMT1A shRNA50	-2,571	-22,67 to 17,53	No	ns	0,9803
CMT1A shRNA49 vs. CMT1A shRNA50	9,476	-9,653 to 28,61	No	ns	0,4828
2 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	55,33	27,23 to 83,43	Yes	***	0,0008
WT scr.-shRNA vs. CMT1A shRNA49	23,81	-3,970 to 51,59	No	ns	0,0949
WT scr.-shRNA vs. CMT1A shRNA50	21,81	-5,967 to 49,59	No	ns	0,1308
CMT1A scr.-shRNA vs. CMT1A shRNA49	-31,52	-46,66 to -16,39	Yes	***	0,0003
CMT1A scr.-shRNA vs. CMT1A shRNA50	-33,52	-48,64 to -18,41	Yes	***	0,0002
CMT1A shRNA49 vs. CMT1A shRNA50	-2,000	-15,23 to 11,23	No	ns	0,9686
3 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	79,45	31,53 to 127,4	Yes	**	0,0034
WT scr.-shRNA vs. CMT1A shRNA49	44,17	-4,010 to 92,34	No	ns	0,0730
WT scr.-shRNA vs. CMT1A shRNA50	35,21	-13,19 to 83,62	No	ns	0,1736
CMT1A scr.-shRNA vs. CMT1A shRNA49	-35,29	-58,81 to -11,76	Yes	**	0,0039
CMT1A scr.-shRNA vs. CMT1A shRNA50	-44,24	-69,13 to -19,35	Yes	**	0,0011
CMT1A shRNA49 vs. CMT1A shRNA50	-8,952	-35,37 to 17,46	No	ns	0,7483
6 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	63,10	22,58 to 103,6	Yes	**	0,0044
WT scr.-shRNA vs. CMT1A shRNA49	25,86	-14,21 to 65,92	No	ns	0,2245
WT scr.-shRNA vs. CMT1A shRNA50	24,19	-17,49 to 65,87	No	ns	0,3375
CMT1A scr.-shRNA vs. CMT1A shRNA49	-37,24	-56,15 to -18,33	Yes	***	0,0009
CMT1A scr.-shRNA vs. CMT1A shRNA50	-38,90	-65,61 to -12,19	Yes	**	0,0050
CMT1A shRNA49 vs. CMT1A shRNA50	-1,667	-26,05 to 22,71	No	ns	0,9958
9 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	70,10	38,41 to 101,8	Yes	***	0,0004
WT scr.-shRNA vs. CMT1A shRNA49	22,71	-11,86 to 57,29	No	ns	0,2560
WT scr.-shRNA vs. CMT1A shRNA50	25,90	-5,965 to 57,77	No	ns	0,1200

CMT1A scr.-shRNA vs. CMT1A shRNA49	-47,38	-73,21 to -21,55	Yes	**	0,0011
CMT1A scr.-shRNA vs. CMT1A shRNA50	-44,19	-63,51 to -24,87	Yes	****	<0,0001
CMT1A shRNA49 vs. CMT1A shRNA50	3,190	-22,96 to 29,35	No	ns	0,9817
12 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	49,57	41,03 to 58,11	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	13,33	4,051 to 22,62	Yes	**	0,0053
WT scr.-shRNA vs. CMT1A shRNA50	8,000	-2,062 to 18,06	No	ns	0,1384
CMT1A scr.-shRNA vs. CMT1A shRNA49	-36,24	-44,17 to -28,31	Yes	****	<0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-41,57	-50,55 to -32,59	Yes	****	<0,0001
CMT1A shRNA49 vs. CMT1A shRNA50	-5,333	-14,99 to 4,320	No	ns	0,3919

Griptest, Strength (N)

Within each row, compare columns (simple effects within rows)	
Number of families	6
Number of comparisons per family	6
Alpha	0,05

Tukey's multiple comparisons test, two-sided	Mean Diff,	95,00% CI of diff,	Significant?	Summary	Adjusted *p*-Value
1 month					
WT scr.-shRNA vs. CMT1A scr.-shRNA	0,7286	-0,3728 to 1,830	No	ns	0,2503
WT scr.-shRNA vs. CMT1A shRNA49	1,081	0,1810 to 1,981	Yes	*	0,0177
WT scr.-shRNA vs. CMT1A shRNA50	1,071	0,1624 to 1,980	Yes	*	0,0199
CMT1A scr.-shRNA vs. CMT1A shRNA49	0,3524	-0,7189 to 1,424	No	ns	0,7558
CMT1A scr.-shRNA vs. CMT1A shRNA50	0,3429	-0,7346 to 1,420	No	ns	0,7742
CMT1A shRNA49 vs. CMT1A shRNA50	-0,009524	-0,8709 to 0,8519	No	ns	>0,9999

2 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	3,900	2,475 to 5,325	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	1,343	0,3521 to 2,334	Yes	**	0,0086
WT scr.-shRNA vs. CMT1A shRNA50	1,195	0,1976 to 2,193	Yes	*	0,0185
CMT1A scr.-shRNA vs. CMT1A shRNA49	-2,557	-3,910 to -1,204	Yes	**	0,0012
CMT1A scr.-shRNA vs. CMT1A shRNA50	-2,705	-4,061 to -1,349	Yes	***	0,0008
CMT1A shRNA49 vs. CMT1A shRNA50	-0,1476	-0,9460 to 0,6507	No	ns	0,9450
3 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	3,019	2,006 to 4,032	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	1,367	0,2981 to 2,435	Yes	*	0,0120
WT scr.-shRNA vs. CMT1A shRNA50	1,267	0,2339 to 2,299	Yes	*	0,0156
CMT1A scr.-shRNA vs. CMT1A shRNA49	-1,652	-2,778 to -0,5263	Yes	**	0,0045
CMT1A scr.-shRNA vs. CMT1A shRNA50	-1,752	-2,847 to -0,6580	Yes	**	0,0023
CMT1A shRNA49 vs. CMT1A shRNA50	-0,1000	-1,242 to 1,042	No	ns	0,9935
6 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	3,229	1,432 to 5,025	Yes	**	0,0015
WT scr.-shRNA vs. CMT1A shRNA49	0,7905	-0,9637 to 2,545	No	ns	0,4874
WT scr.-shRNA vs. CMT1A shRNA50	0,9857	-0,8984 to 2,870	No	ns	0,4288
CMT1A scr.-shRNA vs. CMT1A shRNA49	-2,438	-3,401 to -1,475	Yes	***	0,0001
CMT1A scr.-shRNA vs. CMT1A shRNA50	-2,243	-3,605 to -0,8809	Yes	**	0,0020
CMT1A shRNA49 vs. CMT1A shRNA50	0,1952	-1,055 to 1,446	No	ns	0,9559
9 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	2,881	1,834 to 3,928	Yes	****	<0,0001
WT scr.-shRNA vs. CMT1A shRNA49	0,7857	-0,09970 to 1,671	No	ns	0,0877
WT scr.-shRNA vs. CMT1A shRNA50	0,1952	-0,6762 to 1,067	No	ns	0,9050
CMT1A scr.-shRNA vs. CMT1A shRNA49	-2,095	-3,066 to -1,125	Yes	***	0,0002
CMT1A scr.-shRNA vs. CMT1A shRNA50	-2,686	-3,645 to -1,727	Yes	****	<0,0001
CMT1A shRNA49 vs. CMT1A shRNA50	-0,5905	-1,338 to 0,1566	No	ns	0,1416
12 months					
WT scr.-shRNA vs. CMT1A scr.-shRNA	3,086	1,739 to 4,433	Yes	***	0,0001
WT scr.-shRNA vs. CMT1A shRNA49	0,9238	-0,4650 to 2,313	No	ns	0,2496
WT scr.-shRNA vs. CMT1A shRNA50	1,467	0,1451 to 2,788	Yes	*	0,0286

CMT1A scr.-shRNA vs. CMT1A shRNA49	-2,162	-3,433 to -0,8903	Yes	**	0,0014
CMT1A scr.-shRNA vs. CMT1A shRNA50	-1,619	-2,807 to -0,4307	Yes	**	0,0077
CMT1A shRNA49 vs. CMT1A shRNA50	0,5429	-0,6999 to 1,786	No	ns	0,5802

Supplementary Table S3 List of the shRNAs and primers used and their sequences

Name	Genbank reference	Sequence (5'-3')
ShRNA sh1	[NM_008885.2]	CGCGGTGCTAGTGTTGCTCTT
ShRNA sh1	[NM_008885.2]	CACTGACTACTCCTATGGCTT
ShRNA shA	[NM_000304.4]	CCTGTTCTTCTGCCAACTCTT
ShRNA shB	[NM_000304.4]	GGCAATGGACACGCAACTGAT
Genotyping primers	[NM_008885.2]	Forward: GACAAACCCCAGACAGTTG Reverse: CCAGAAAGCCAGGGAACTC
ITR-2 primers		Forward: GGAACCCCTAGTGATGGAGTT Reverse: CGGCCTCAGTGAGCGA Taqman Probe used for vector titer: FAM- CACTCCCTCTCTGCGCGCTCG-BBQ
Rat-specific *Pmp22*	[NM_17037.1]	Forward: GACAAACCCCAGATGGCC Reverse: CCGCAGCCACCAGCTATTGGT
Mouse-specific *Pmp22*	[NM_008885.2]	Forward: GACAAACCCCAGACAGTTGA Reverse: CAGGAGCCACCAGCTATTACT
Total *Pmp22* (mouse and rat)		Forward: TGTACCACATCCGCCTTGG Reverse: GAGCTGGCAGAAGAACAGGAAC
Rat *Rsp9*	[NM_031108.2]	Forward: ATCCGCCAACGTCACATC Reverse: CCGCCACCATAAGGAGAAC
Rat *Actb*	[NM_031144.3]	Forward: CACCATGTACCCAGGCATT Reverse: ACTTGCGCTCAGGAGGAG
Rat *Mpz*	[NM_017027.1]	Forward: TGTTGCTGCTGTTGCTCTTC Reverse: TTGTGAAATTTCCCCTTCTCC
GFP		Forward: ACTACAACAGCCACAACGTCTATATCA Reverse: GGCGGATCTTGAAGTTCACC Taqman Probe used for Biodistribution study: FAM-CCGACAAGCAGAAGAACGGCATCA-TAMRA
Rat *Hprt1*	[NM_012583.2]	Forward: GCGAAAGTGGAAAAGCCAAGT Reverse: GCCACATCAACAGGACTCTTGTAG Taqman Probe used for biodistribution study: JOE-CAAAGCCTAAAAGACAGCGGCAAGTTGAAT- TAMRA
Rat *Gstt2*	[NM_012796.2]	ForwarGCTCTACCTGGACCTGCTGT Reverse: GAATTGCTCGCTCAAGTGCT
Rat *Ctsa*	[NM_001011959.2]	Forward: TTACAGAGCACGGTCCCTTC Reverse: ATATACAGCATGTTGGCAATCAG
Rat *Nrg1-I*	[NM_001271125.2]	Forward: GGGAAGGGCAAGAAGAAGG Reverse: TTTCGCACCGGAGCACTAGC
Rat *Pparg*	[NM_013124.3]	Forward: GGTGAAACTCTGGGAGATCCT Reverse: AATGGCATCTCTGTGTCAAC
Rat *Fn3krp*	[NM_001107077.2]	Forward: CATTCAGCCCCAGATGGA Reverse: CACGGAACAGGTCAGGAATC
Rat *Cda*	[NM_001108688.1]	Forward: AGCCTGCCGACAAGTCAT Reverse: AACCTTCAGTGAATCTTCTGCAA
Rat *Enpp1*	[NM_053535.1]	Forward: TACCCCAAGTCATCCCAAAG Reverse: AAGTCCATGATCGGCACAAT
Rat *Gria1*	[NM_031608.1]	Forward: AGGGATCGACATCCAGAGAG Reverse: TGCACATTTCCTGTCAAACC
Rat *Anpep*	[NM_031012.1]	Forward: GCCCACCTGGAATCTGAA Reverse: CTGGCGTGTTGACCTCGT