



**Supplementary Figure S3.** Photopic b-wave amplitudes of treated mutant mice and histological analysis of the periphery following 7m8.hCLN3 treatment. Photopic b-wave amplitudes of *Cln3*<sup>Δex7/8</sup> mice that received intravitreal injections with 7m8.CMV.hCLN3 (**A**) and 7m8.Grm6.hCLN3 (**B**) at 12 and 15 months. 7m8.CMV.hCLN3 treatment resulted in significantly increased photopic b-wave amplitudes at 15 months, while 7m8.Grm6.hCLN3 treatment did not lead to higher b-wave amplitudes. Wild-type recordings from Fig. 1 were added as a reference. For *n* numbers see Figs. 3 and 4, respectively. Two-way ANOVA with Bonferroni test (\*\**p* < 0.01). (**C**) Representative images of the peripheral retina of untreated, 7m8.CMV.hCLN3-, and 7m8.Grm6.hCLN3-treated mutant mice at 15 months. The number of PKC $\alpha$ -positive cells is increased in the treated compared with the untreated retinas. Scale bar: 25  $\mu$ m. hCLN3, human CLN3.

**Supplementary Table S1.** Summary of scotopic b-wave amplitudes in *Cln3*<sup>Δex7/8</sup> mice that received intravitreal and subretinal injection

Mouse strain	AAV treatment	Titer (vg/eye)	Scotopic b-wave amplitudes (mean $\pm$ SD)	
			12 Months	15 Months
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.CMV.Cln3-ires-eGFP	1 $\times$ 10 <sup>9</sup>	281.2 $\pm$ 48.4	192.3 $\pm$ 40.4
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.CMV.Cln3-ires-eGFP	1 $\times$ 10 <sup>10</sup>	278 $\pm$ 65.1	283.9 $\pm$ 52.5
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.CMV.hCLN3	1 $\times$ 10 <sup>10</sup>	253 $\pm$ 51.3	251.4 $\pm$ 73.1
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.Grm6.hCLN3	1 $\times$ 10 <sup>9</sup>	204.5 $\pm$ 60	186.3 $\pm$ 67.4
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.Grm6.hCLN3	1 $\times$ 10 <sup>10</sup>	239.2 $\pm$ 60	206.6 $\pm$ 121
<i>Cln3</i> <sup>Δex7/8</sup>	7m8.CMV.eGFP	1 $\times$ 10 <sup>10</sup>	174.2 $\pm$ 41.1	99.7 $\pm$ 27
<i>Cln3</i> <sup>Δex7/8</sup>	AAV8.CMV.mCln3-ires-eGFP	1 $\times$ 10 <sup>10</sup>	175.2 $\pm$ 54.5	109.3 $\pm$ 55.1
<i>Cln3</i> <sup>Δex7/8</sup>	AAV8.CMV.eGFP	1 $\times$ 10 <sup>10</sup>	172 $\pm$ 61.5	93 $\pm$ 40.6
<i>Cln3</i> <sup>Δex7/8</sup>	—	—	154.5 $\pm$ 37.4	79.9 $\pm$ 31.9
Wild type	—	—	327.6 $\pm$ 64	249.5 $\pm$ 51.3

Overview of the mean  $\pm$  SD of the scotopic b-wave amplitudes (at 1 cd.s/m<sup>2</sup>) in all AAV-treated mutant mice over time. Untreated mutant and wild-type controls were added as a reference from Fig. 2.

AAV, adeno-associated virus; CMV, cytomegalovirus; eGFP, enhanced green fluorescent protein; hCLN3, human CLN3; ires, internal ribosome entry site; SD, standard deviation.