

Supplementary Figure S2. ERG recordings and retinal histology of treated mutant eyes. (A) Photopic b-wave amplitudes of $Cln3^{\Delta ex7/8}$ following intravitreal injections with 7m8.CMV.mCln3-ires-eGFP and subretinal injections with AAV8.CMV.mCln3-ires-eGFP. Wild-type recordings from Fig. 1 were added as a reference. For *n* numbers see Fig. 2. Two-way ANOVA with Bonferroni test (*p < 0.05, ***p < 0.001). (B) Representative confocal images of the peripheral retina from untreated, 7m8.CMV.eGFP-, and 7m8.CMV.mCln3-ires-eGFP-treated retinas at 15 months. PKC α immunostaining demonstrates that the intravitreal delivery of mCln3 also had a therapeutic effect on the number of bipolar cells in the periphery compared with untreated or control-treated mutant retinas. (C) Representative confocal images of the midretina from AAV8.CMV.mCln3-ires-eGFP-treated retinas at 15 months. Immunostaining for eGFP reveals transduced photoreceptors. Staining for PKC α indicates that the treatment did not prevent the loss of rod bipolar cells, which is in line with the reduced ERG b-wave amplitudes in AAV8-treated retinas. Scale bar: 25 μ m. CMV, cytomegalovirus; eGFP, enhanced green fluorescent protein; PKC α , protein kinase C alpha-subunit.