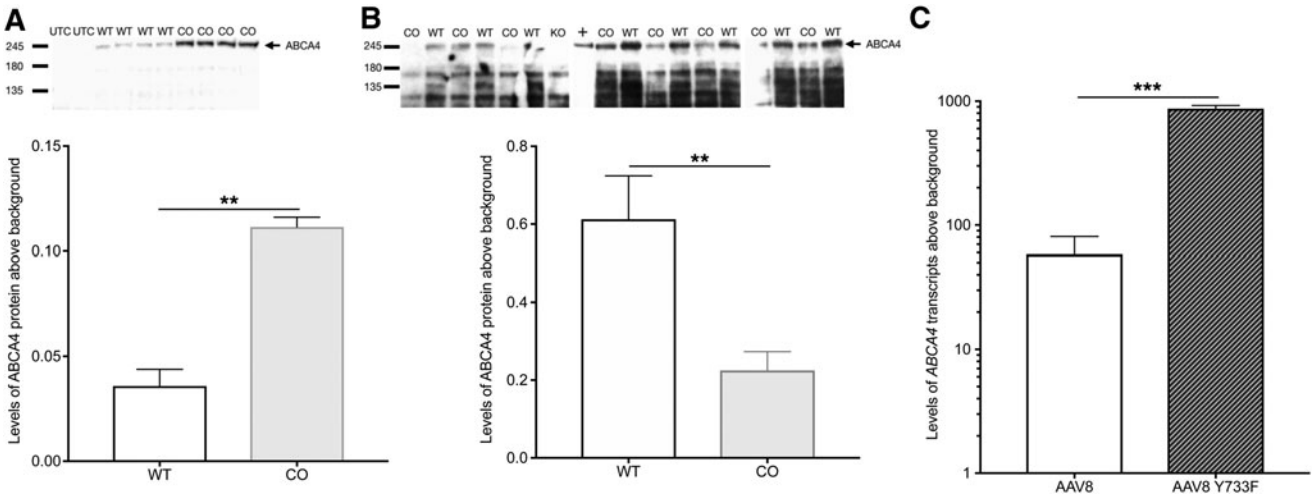
Supplementary Data



Supplementary Figure S1. Comparisons of ATP-binding cassette transporter protein family member 4 (ABCA4) expression levels following treatment with wild-type (WT) or codon-optimized (CO) *ABCA4* coding sequence. **(A)** Otherwise identical plasmids containing WT or CO *ABCA4* coding sequence were used to transfect HEK293T cells with a significant difference in subsequent ABCA4 protein levels determined (unpaired *t*-test, $n=4$, $^{***}p=0.0002$). **(B)** *Abca4*$^{-/-}$ mice received subretinal injection of AAV2/8 overlapping dual vectors containing either WT or CO *ABCA4* coding sequence. At 6 weeks postinjection, WT injected eyes had significantly more ABCA4 than CO injected eyes (paired *t*-test, $n=8$, $^{**}p=0.002$). **(C)** Levels of *ABCA4* mRNA transcripts were assessed by quantitative PCR from *Abca4*$^{-/-}$ retinae injected with AAV2/8 or AAV2/8 Y733F dual vectors carrying identical transgenes (Mann–Whitney test, $n=4$, $^{***}p=0.0002$). Error bars represent SEM. ABCA4, ATP-binding cassette transporter protein family member 4; CO, samples treated with transgenes containing codon-optimized *ABCA4* coding sequence; KO, uninjected *Abca4*$^{-/-}$ retina lysate; UTC, untransduced HKE293T cell lysate; WT, samples treated with transgenes containing wild-type *ABCA4* coding sequence; +, *ABCA4* transfected HEK293T cell lysate.