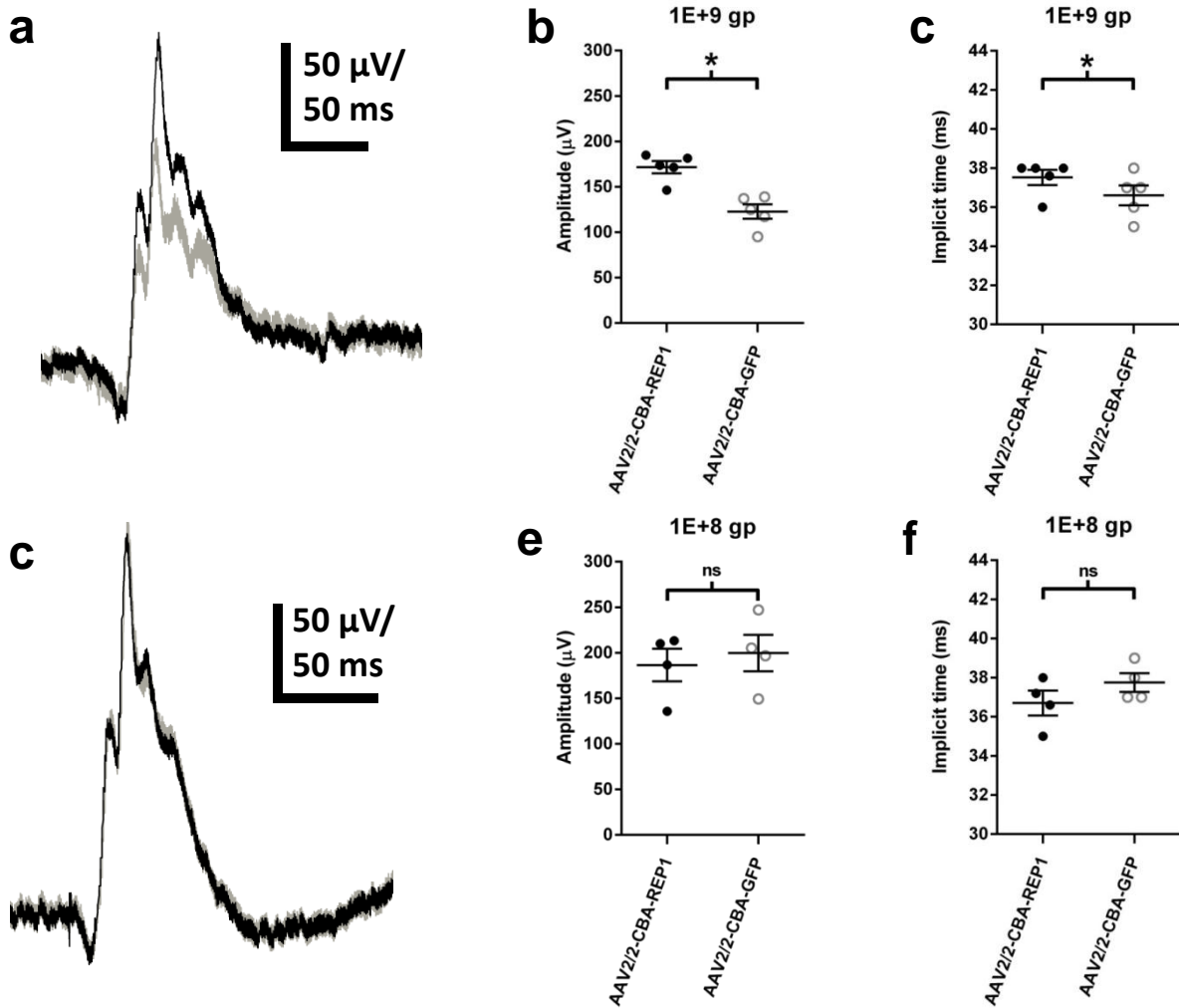


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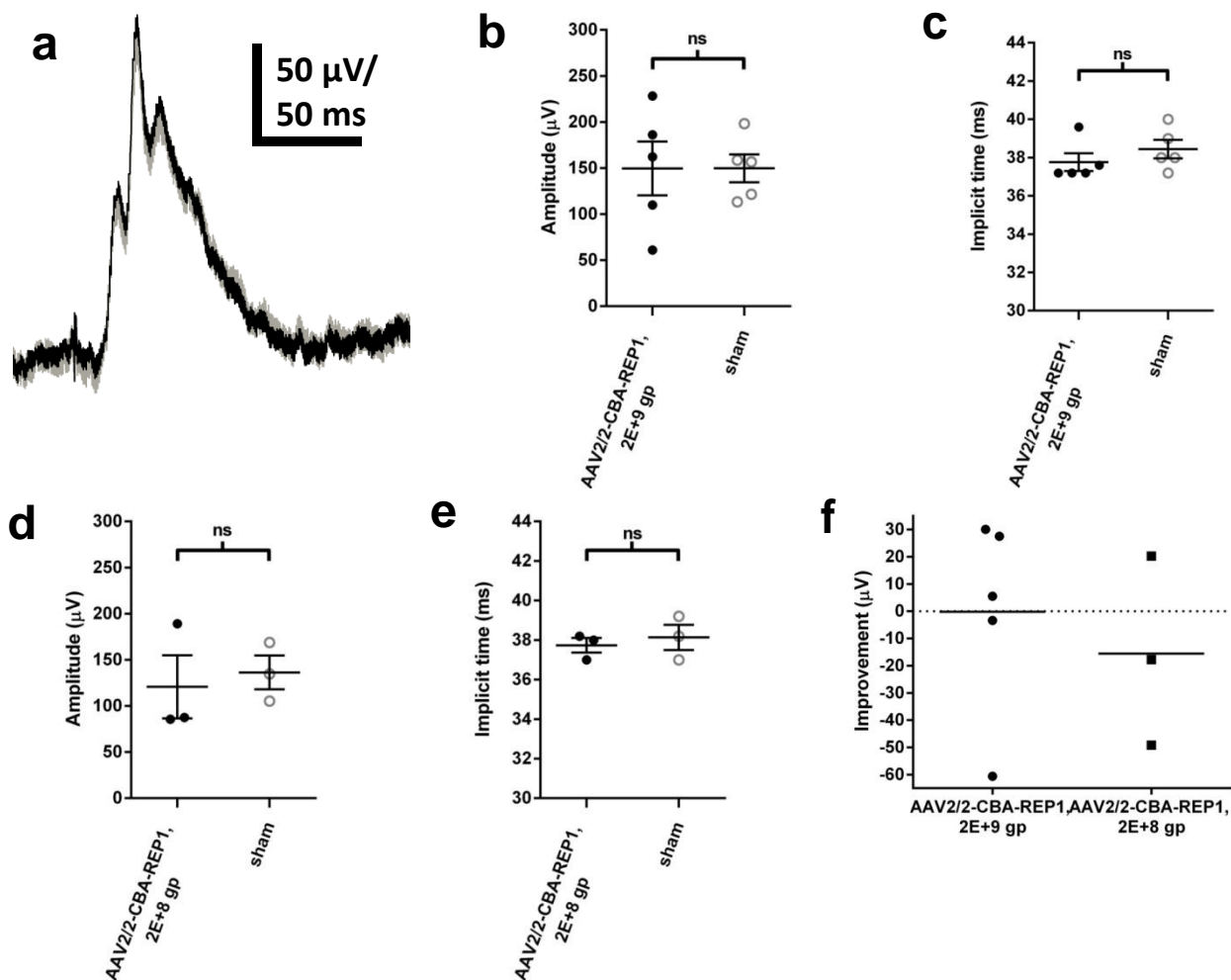
Electronic Supplementary Material

**Functional expression of Rab Escort Protein 1 following AAV2-mediated gene delivery in the retina of choroideremia mice and human cells *ex vivo***

Tanya Tolmachova, Oleg E. Tolmachov, Alun R. Barnard , Samantha R. de Silva, Daniel M. Lipinski, Nathan J. Walker, Robert E. MacLaren, Miguel C. Seabra



**Supplementary Figure S1.** Light-adapted ERG analysis of wild type mice treated with AAV2/2-CBA-REP1 and AAV2/2-CBA-GFP. **a** Representative averaged ERG traces from the eyes injected with high dose ( $1 \times 10^9$  gp) AAV2/2-CBA-REP1 (shown in black) and AAV2/2-CBA-GFP (shown in grey). **b** Quantification of b-wave amplitude in high dose ( $1 \times 10^9$  gp) AAV2/2-CBA-REP1 (filled black circles) and AAV2/2-CBA-GFP (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=5$ . \* denotes significant difference ( $p < 0.05$ ) between group means (paired t test,  $t_4=4.035$ ,  $p=0.0157$ ). **c** Quantification of b-wave implicit time in high dose ( $1 \times 10^9$  gp) AAV2/2-CBA-REP1 (filled black circles) and AAV2/2-CBA-GFP (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=5$ . \* denotes significant difference ( $p < 0.05$ ) between group means (paired t test,  $t_4=3.570$ ,  $p=0.0234$ ). **d** Representative averaged ERG traces from the eyes injected with the low dose ( $1 \times 10^8$  gp) of AAV2/2-CBA-REP1 (shown in black) and AAV2/2-CBA-GFP (shown in grey). **e** Quantification of b-wave amplitude in low dose ( $1 \times 10^8$  gp) AAV2/2-CBA-REP1 (filled black circles) and AAV2/2-CBA-GFP (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=4$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_3=0.7842$ ,  $p=0.4901$ ). **f** Quantification of b-wave implicit time in low dose ( $1 \times 10^8$  gp) AAV2/2-CBA-REP1 (filled black circles) and AAV2/2-CBA-GFP (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=4$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_3=0.9523$ ,  $p=0.4112$ ).



**Supplementary Figure S2.** Light-adapted ERG analysis of *Chm*<sup>null/WT</sup> mice, treated with AAV2/2-CBA-REP1 and DMEM (sham). **a** Representative averaged ERG traces from high dose ( $2 \times 10^9$  gp) AAV2/2-CBA-REP1 (shown in black) and sham (shown in grey) injected eyes. **b** Quantification of b-wave amplitude in high dose ( $2 \times 10^9$  gp) AAV2/2-CBA-REP1 (filled black circles) and sham (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=5$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_4=0.009733$ ,  $p=0.9927$ ). **c** Quantification of b-wave implicit time in high dose ( $2 \times 10^9$  gp) AAV2/2-CBA-REP1 (filled black circles) and sham (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=3$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_4=1.176$ ,  $p=0.3048$ ). **d** Quantification of b-wave amplitude in low dose ( $2 \times 10^8$  gp) AAV2/2-CBA-REP1 (filled black circles) and sham (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=3$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_2=0.5695$ ,  $p=0.6265$ ). **e** Quantification of b-wave implicit time in low dose ( $2 \times 10^8$  gp) AAV2/2-CBA-REP1 (filled black circles) and sham (open grey circles) injected eyes. Plotted symbols show individual values, horizontal bars show means with  $\pm$ -SEM error bars,  $n=3$ . ns denotes non-significant difference ( $p > 0.05$ ) between group means (paired t test,  $t_2=0.5695$ ,  $p=0.6265$ ). **f** Change in b-wave amplitude in high dose ( $2 \times 10^9$  gp, filled black circles) and low dose ( $2 \times 10^8$  gp, open grey circles) AAV2/2-CBA-REP1 treatment groups relative to contralateral sham treated eye. Plotted symbols show individual values, horizontal bars show means.