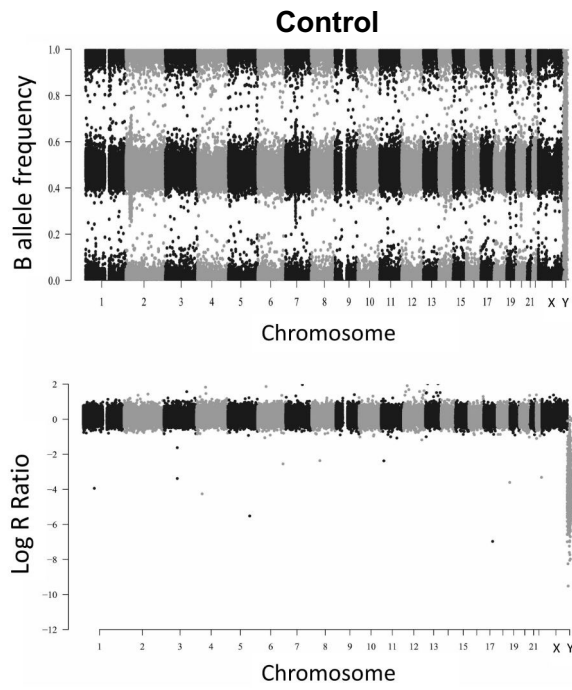
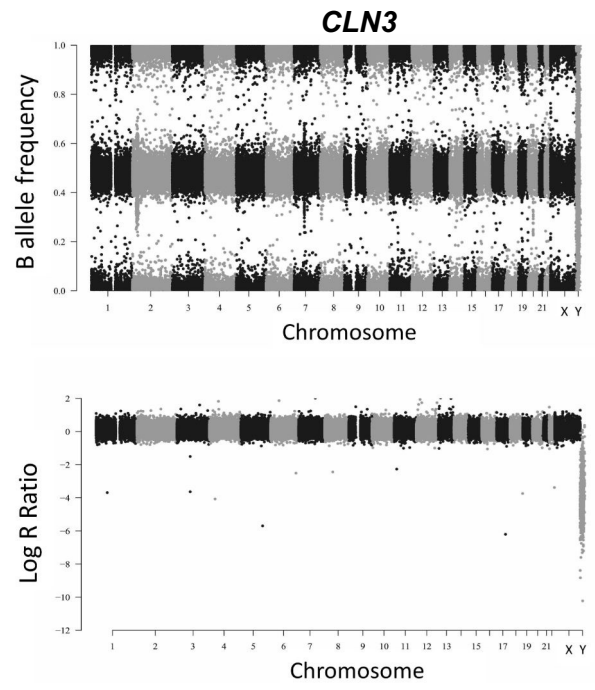
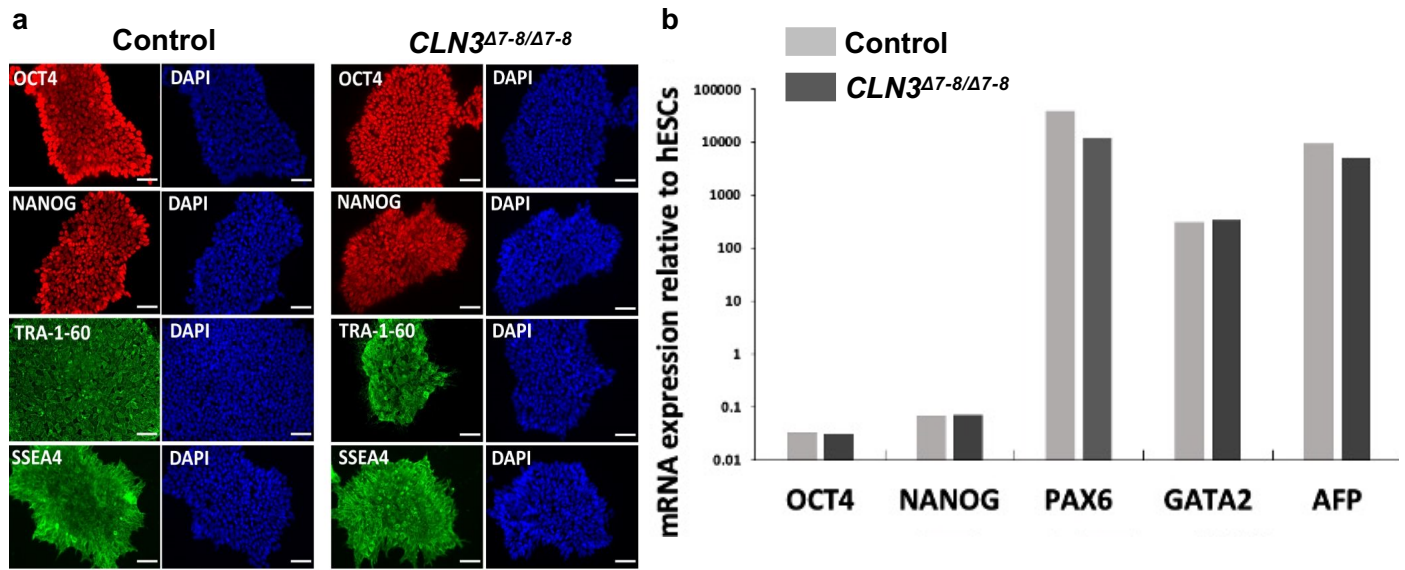


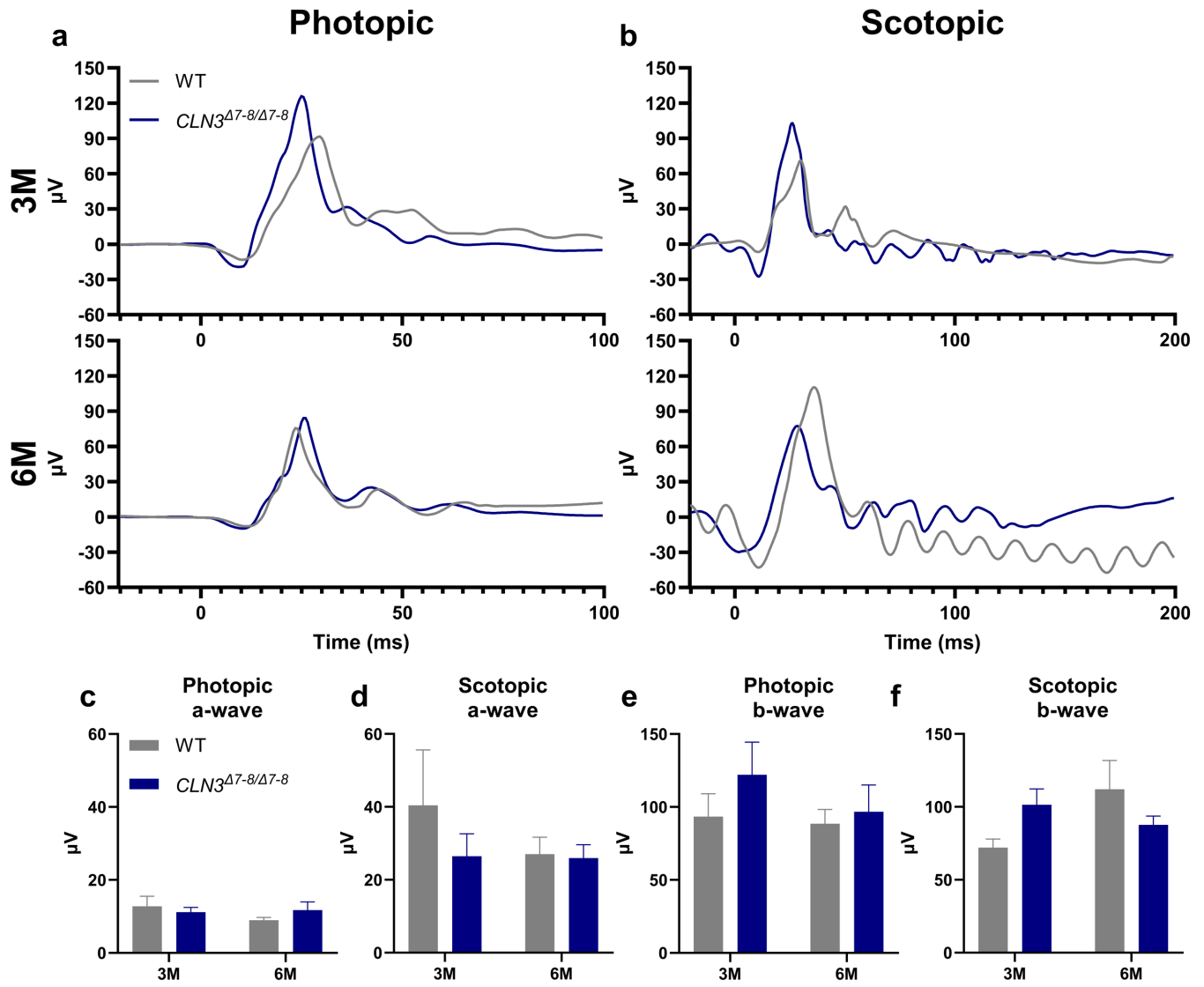
a

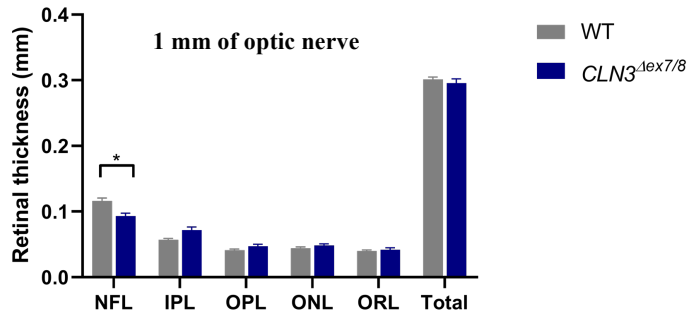
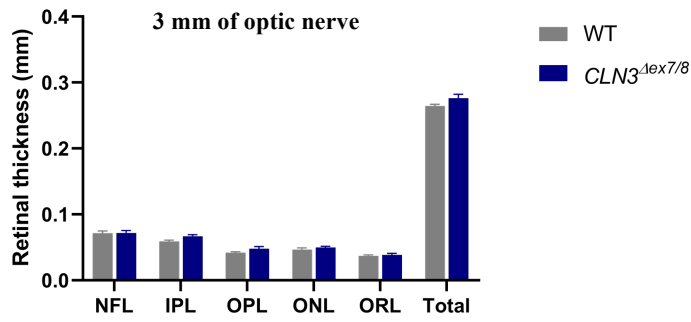
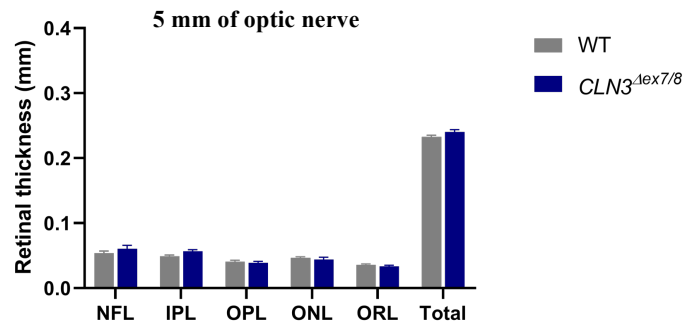


b







a**b****c**

Supplementary Figure Legends

Figure S1. Copy number variation analysis of *CLN3* ^{$\Delta 7-8/\Delta 7-8$} H9 isogenic cell lines.

a, b) Top and bottom panels show B allele frequency (BAF) and Log R ratio (LRR) respectively for the control (a) and *CLN3* ^{$\Delta 7-8/\Delta 7-8$} (b) H9 human embryonic stem cell lines (hESC).

Figure S2. Pluripotency characterization of isogenic *CLN3* ^{$\Delta 7-8/\Delta 7-8$} H9 hESCs.

a) Fluorescence microscopy images showing presence of pluripotency markers OCT4, NANOG, TRA-1-60 and SSEA4 in control and *CLN3* ^{$\Delta 7-8/\Delta 7-8$} hESCs (Scale bar = 100 μ m).

b) Graphical representation of gene expression levels of the pluripotency markers *OCT4* and *NANOG* and the germ layer markers *PAX6* (ectoderm), *GATA2* (mesoderm) and *AFP* (endoderm) in isogenic *CLN3* ^{$\Delta 7-8/\Delta 7-8$} embryoid bodies (EBs) relative to hESCs that were normalized to *EEF2* gene according to the $\Delta\Delta$ Ct method.

Figure S3. Scotopic and photopic ERG measurements in wild-type (WT) versus *CLN3* miniswine at 3 and 6 months of age.

a, b) Representative photopic (a) and scotopic (b) ERG traces of WT (grey) and *CLN3* (blue) miniswine at 3 and 6 months of age.

c, d) Graphical representation of ERG measurements showing amplitudes of photopic a-wave (c) and scotopic a-wave (d) in WT and *CLN3* miniswine retina at 3 (3M) and 6 months (6M) of age.

e, f) Graphical representation showing similar amplitudes of photopic b-wave (e) and scotopic b-wave (f) in WT and *CLN3* miniswine retina at 3 (3M) and 6 months (6M) of age.

Note that for panels c-f, wave recordings from each eye were obtained and analyzed. Absolute values for a-wave amplitudes are displayed. Amplitudes for b-wave displayed as the difference

between b-wave and a-wave peaks. Photopic: 8.0 cd s/m² flash at 2.0 Hz (cone predominant). Scotopic: 8.0 cd s/m² flash at 0.1 Hz (bright flash standard combined response-mixed rods and cones).

Figure S4. OCT analyses of retina thickness in WT versus *CLN3* miniswine at 6 months of age.

a-c) Quantitative analyses of retina thickness at a distance of 1mm (a), 3 mm (b) and 5 mm (c) from the optic nerve showed similar retinal thickness of individual retina cell layers between WT and *CLN3* miniswine at 6 months of age with the exception of nerve fiber layer (NFL) that was slightly thinner in *CLN3* miniswine retina at 1mm from the optic nerve. Individual parameters measured included thickness of total retina, outer nuclear layer (ONL), NFL, inner plexiform layer (IPL), outer plexiform layer (OPL) and outer retina layer (ORL). * $p \leq 0.05$