

Supporting Figure 10. Similar lipofuscin granules accumulation in the retina of Abca4-/-mice independently of the AAV control vector genome size.

Quantification of lipofuscin granules in Abca4-/- mice injected subretinally with dual AAV trans-splicing and hybrid AK control vectors with a combined (5'-half+3'-half) large (8.9 Kb, dual AAV large) or small (2.7-2.9 Kb, dual AAV small) genome [dose of each vector/eye: 1.2×10^9 genome copies (GC)]. Eyes were harvested 2 months post-injection and lipofuscin granules were counted in at least 30 fields ($25 \mu m^2$) for each sample. The number (n) of eyes analyzed is depicted below each bar. Dual AAV small includes Abca4-/- eyes injected with either trans-splicing small (n=2) or hybrid AK small (n=2; total n=4); dual AAV large includes Abca4-/- eyes injected with either trans-splicing large (n=2) or hybrid AK large (n=2; total n=4). Values are represented as mean \pm s.e.m. (standard error of the mean). No statistically significant differences were found using the Student's t-test (p=0.54).