Supporting Figure 11. Subretinal administration of dual AAV trans-splicing and hybrid AK vectors results in MYO7A expression in photoreceptors.

Representative Western blot analysis of sh1-/- retinas 2 months following subretinal delivery of dual AAV trans-splicing (TS) and hybrid AK (AK) vectors encoding for MYO7A-HA under the control of the photoreceptor-specific Rhodopsin promoter (RHO PROMOTER). The arrow indicates full-length murine endogenous Myo7a in sh1+/+ retinas and recombinant human MYO7A expressed from dual AAV vectors in sh1-/- retinas; the molecular weight ladder is depicted on the left; whole retina lysates were loaded in each lane. The picture is representative of: n=4 sh1-/- retinas treated with TS; n=5 sh1-/- retinas treated with dual hybrid AK. TS: retinas injected with dual AAV TS vectors; AK: retinas injected with dual hybrid AK vectors; neg: retinas injected with the 5’-half of either the dual AAV TS or hybrid AK vectors, as negative controls; α-Myo7a: Western blot with anti-Myosin7a antibody; α-Dysferlin: Western blot with anti-Dysferlin antibody, used as loading control.