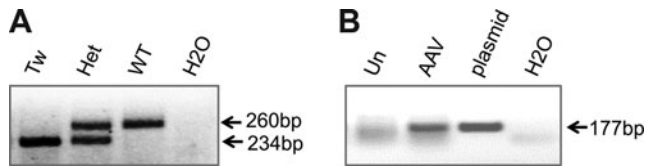
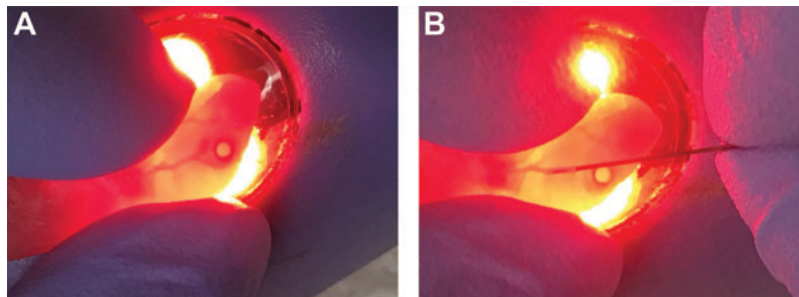


Supplementary Data



Supplementary Fig. S1. PCR genotyping of experimental mice and PCR confirmation of AAV injected twitcher mice. **(A)** Representative PCR photomicrograph of genotyping results. **(B)** Representative PCR photomicrograph of AAV injection validation. Tw, twitcher mouse; Het, heterozygous mouse; WT, wild type mouse; H2O, negative control (without genome DNA); Un, un-injected twitcher mouse; AAV, AAV injected twitcher mouse; Plasmid, positive control using the cis-plasmid pXP46 as the PCR template; H2O, negative control.



Supplementary Fig. S2. Systemic injection of the AAV vector via the facial vein in a 2-day-old twitcher mouse. **(A)** Identification of the facial vein using the Wee-sight transilluminator vein finder. **(B)** Delivery of the AAV vector from the facial vein.
