

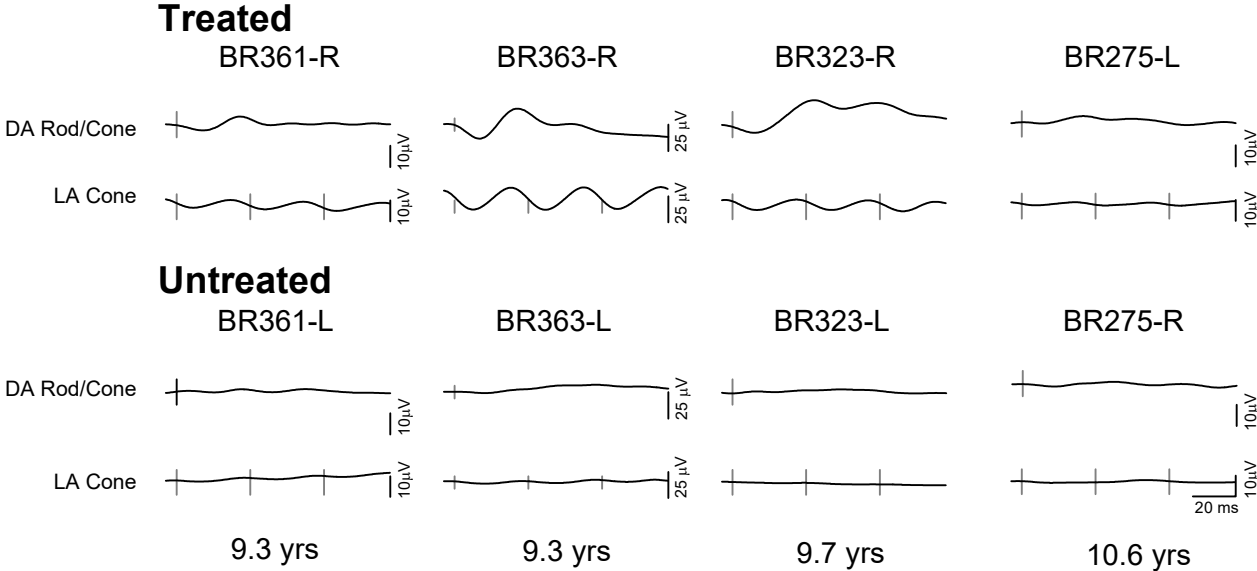
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Supplemental Information

Long-Term Structural Outcomes of Late-Stage *RPE65* Gene Therapy

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Supplemental Fig. 1



Supplemental Figure 1. Illustration of full-field ERGs recorded from the treated and untreated eyes of RPE65-mutant dogs at the end of study. Shown are the dark-adapted mixed rod-cone responses (DA Rod/Cone) and the light-adapted 29Hz flicker responses (LA Cone). In contrast to untreated eyes, at the end of study all treated eyes had distinct ERG responses under dark- and light-adapted conditions. There was, however, variability in the ERG amplitudes among the treated eyes with BR363-R showing the most robust responses, and BR275-L showing the most reduced responses. There were no recordable responses in untreated eyes.

Supplemental Table 1. Experimental animals and procedures used in the study.

Animal ID	Gender	Status	Tx Age yrs	Vector vol and titer* (dose)	OCT age yrs	Histopathology (PI interval) yrs
BR275	Female	affected		OD: --	6.4, 7.2, 7.5, 7.9, 8.9, 9.4, 10, 10.6 (OS only; cataract OD)	10.6 (4)
			6.6	OS: 190 μ L; 3.96×10^{11} (7.5×10^{10} vg)		
BR323	Female	affected	5.3	OD: 200 μ L; 3.96×10^{11} (7.9×10^{10} vg)	5.1, 5.6, 6.0, 6.3, 7.3, 7.8, 8.4, 9.1, 9.7 (OU)	9.9 (4.6)
				OS: --		
BR361	Male	affected	4.9	OD: 160 μ L; 3.96×10^{11} (6.3×10^{10} vg)	4.8, 5.2, 5.6, 5.9, 6.9, 7.4, 8.0, 8.7, 9.4 (OU)	9.5 (4.6)
				OS: --		
BR363	Female	affected	4.9	OD: 200 μ L; 3.96×10^{11} (7.9×10^{10} vg)	4.8, 5.2, 5.5, 5.9, 6.9, 7.4, 8.0, 8.7, 9.4 (OU)	9.5 (4.6)
				OS: --		
BR304	Female	affected	-	--	OU-5.2	5.2
BR318	Female	affected	-	--	OU-5.1, 5.2	5.2
BR354	Female	affected	-	--	OS-5.0	-
CGBCAN, CGBCDI, CGBGS	Male Male Male	WT	-	--	OU-0.7	-
X236	Female	WT	-	-	-	8.6
GSK-1	Male					4.3
GSK2	Male					4.3
D347	Male					6.0

* vector: rAAV2-CMV/CBA-hRPE65; vector titer expressed as vector genomes/mL
 affected=homozygous for *RPE65* mutation; WT=wildtype control
 PI=post-injection; OS= left eye; OD=right eye; OU=both eyes