

Supplementary Table 2. Testing vector-promoter combinations for targeted cell-specific expression in the canine model; efficacy and specificity of the same vector-promoter in other species.

Target cell and Disease	Vector	Promoter	Reporter	Dog	Mouse	Non human primate
RPE <i>BEST1</i> -BVMD	AAV2	hVMD2 ¹	<i>GFP</i> , <i>cBEST1</i> , <i>hBEST1</i>	Specific RPE expression ¹		
	AAV1	hVMD2	<i>GFP</i> , <i>cBEST1</i> , <i>hBEST1</i>	Specific RPE expression. Cone toxicity with <i>BEST1</i> expression ¹		
Cones <i>CNGB3</i> -ACHM Study 1 ²						
<u>L/M-cones</u>	AAV5	PR0.5 ----- 3LCR-PR0.5 -----	<i>GFP</i>	No cone expression ----- Limited and weak cone transduction -----		
		PR2.1 ³ -----		Robust expression limited to L/M-cones -----		
<u>S-cones</u>		HB569 ⁴		L/M-cones, rods, RPE		
Study 2 ⁵	AAV5	PR2.1, PR1.7, PR1.5, PR1.1	<i>GFP</i>		Expressed in cones and rods. RPE expression with all except PR1.7 ⁵	PR1.7: expression limited to L/M- and S-cones PR2.1: expression in L/M-cones and ± in S-cones.
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		hIRBP/GNAT2				none
	AAV2 _{tYF-}	PR1.7	<i>GFP</i>			Expression limited to L/M- and S- cones
		PR2.1				Expression in L/M-cones and \pm in S-cones.
Rods						
<i>RHO</i> -ADRP ⁶	AAV5	mOP	<i>GFP</i>	rods		
		hGRKI	<i>GFP</i>	rods		
		CBA	<i>GFP</i>	RPE, rods, cones		
Rods and cones						
<i>RPGR</i> -XLRP Study 1 ⁷	AAV5	hIRBP	<i>GFP</i>	Rods and cones	Rods and cones (Lewin et al., unpublished)	
Study 2 ⁸	AAV5	hIRBP	<i>GFP</i>			Rods
		hGRK1				Rods and cones (foveal and peripheral)
Study 3 ⁹	AAV5	hIRPB	<i>hRPGRstb</i>		Rods and cones	
<i>RPGRIP1</i> -CORD ¹⁰	AAV5	RK	<i>eGFP</i>	Rods and L/M cones		

hRPGRstb: human RPGR sequence stabilized for plasmid propagation, vector production and *in vivo* expression⁹

References

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