

Supplemental Information

Otx2-Genetically Modified Retinal Pigment

Epithelial Cells Rescue Photoreceptors

after Transplantation

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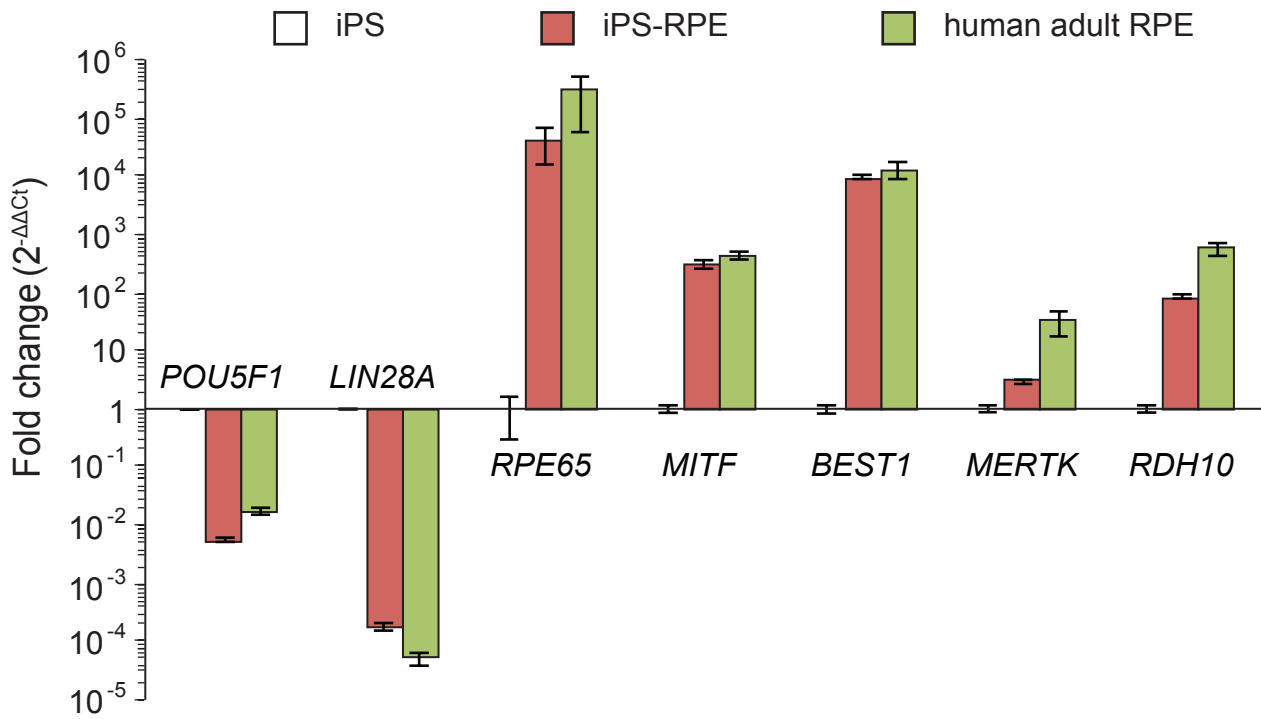


Figure S1 Fold change in expression by qRT-PCR of RPE specific markers in differentiated human iPS-derived RPE and human adult RPE cells as compared to undifferentiated human iPS cells. All data were normalized to 18S rRNA. Mean with SD.

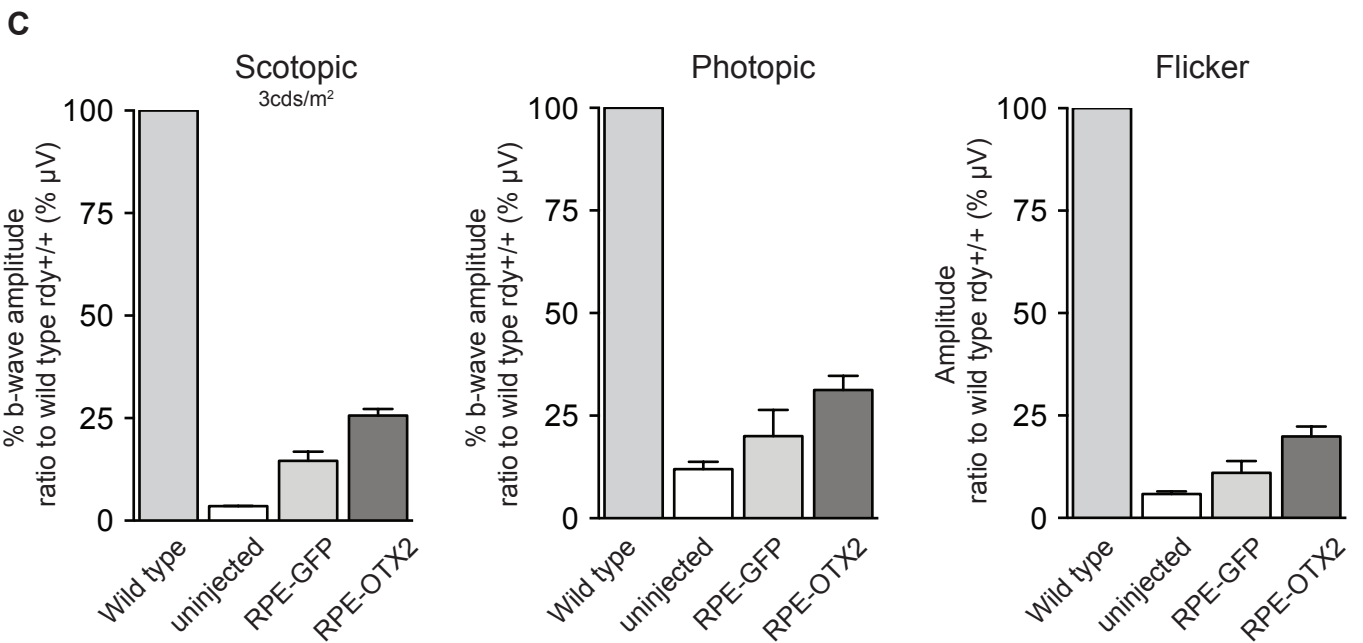
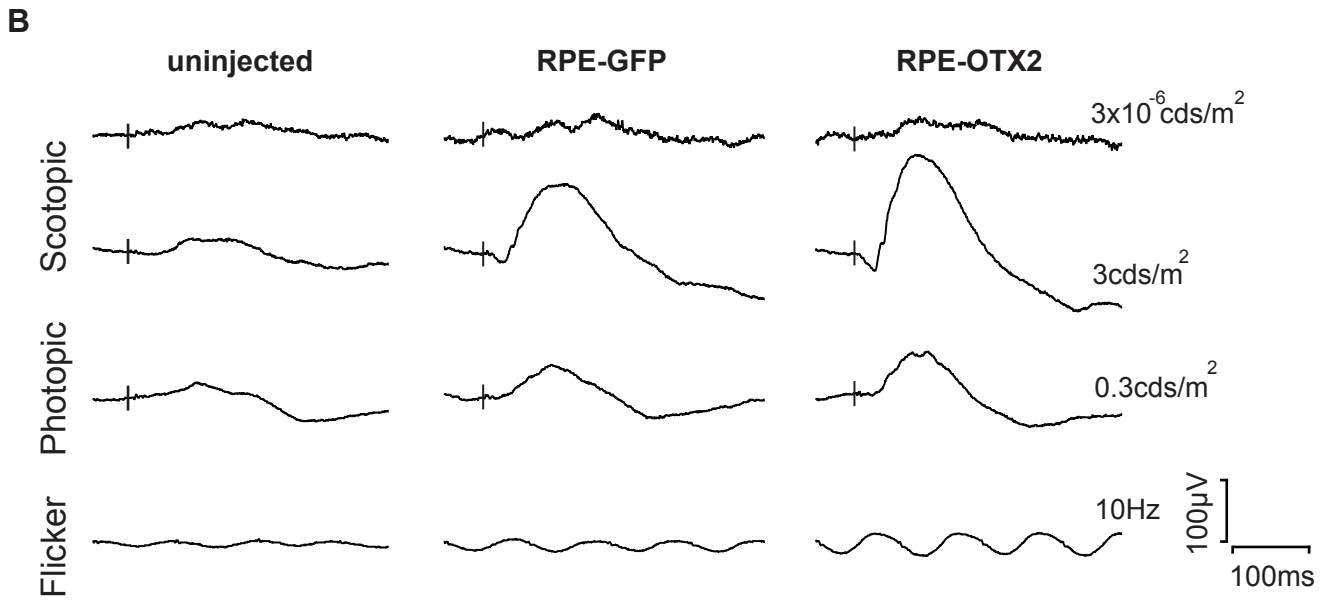
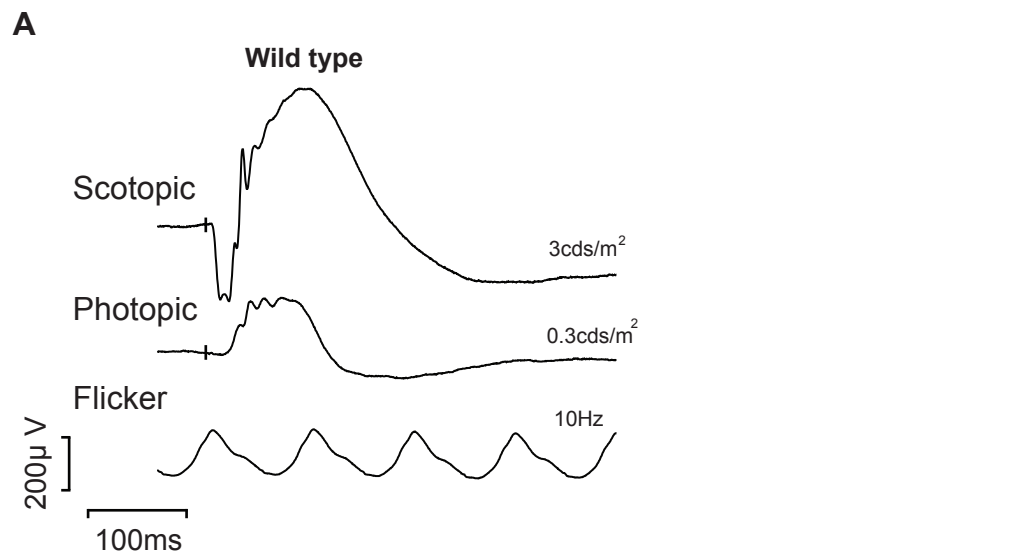


Figure S2. ERG amplitude comparisons in the uninjected and transplanted RCS rat eyes. (A) Characteristic ERG traces of wild type RCS rat. **(B)** Characteristic ERG amplitudes recorded at 42 post-injection days in dystrophic uninjected, RPE-GFP and RPE-OTX2 grafted animals. **(C)** Percentage of signal relative to wild type ERG recording comparison..

A

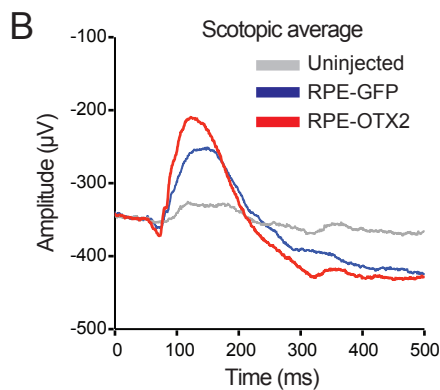
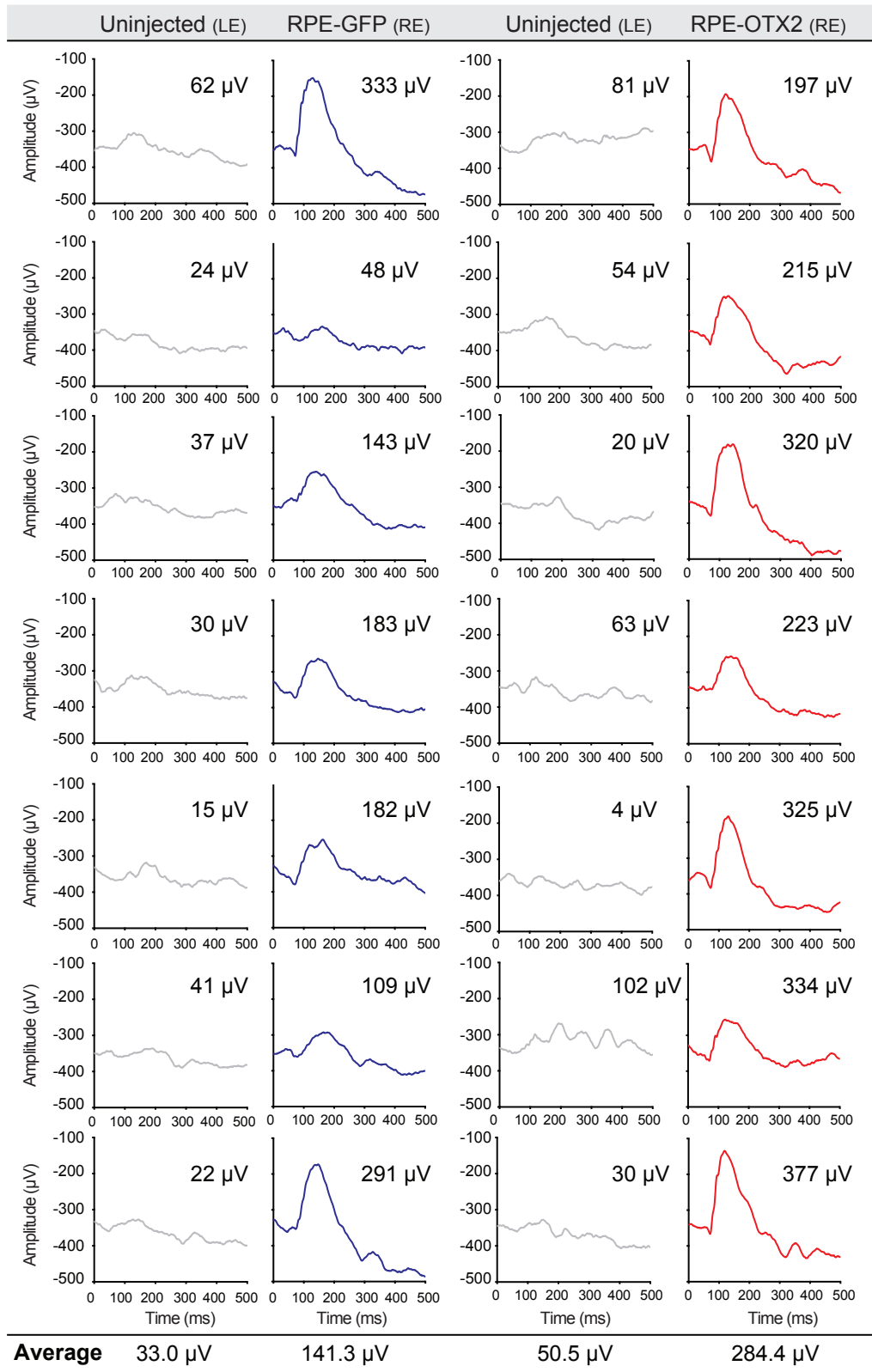
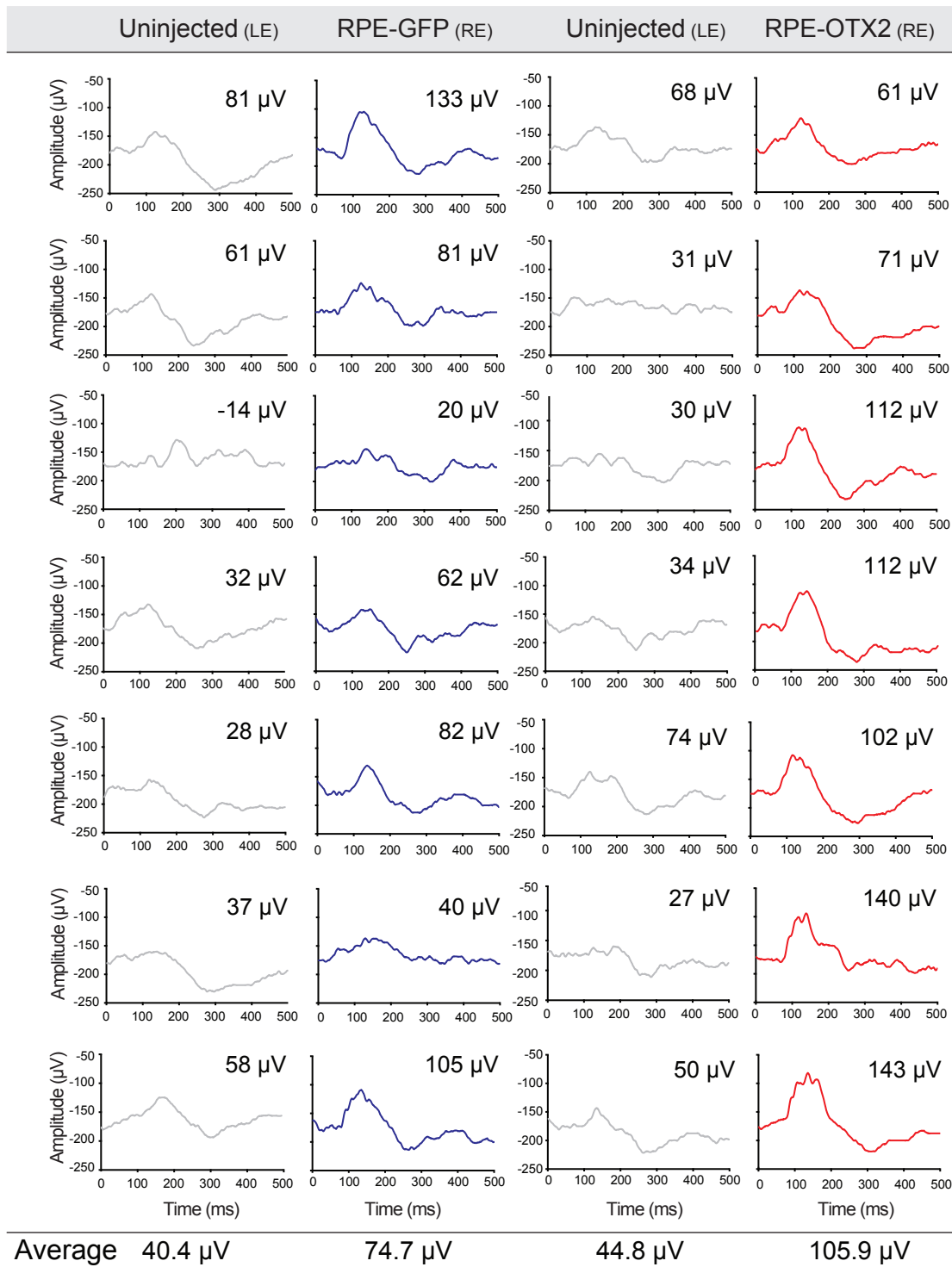


Figure S3. Scotopic ERG recording of the transplanted RCS rats. (A) Individual traces. **(B)** Averaged traces. LE: left eye and RE: right eye.

A



B

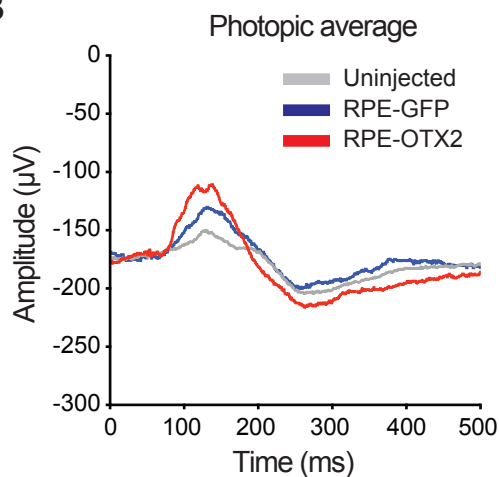


Figure S4. Photopic ERG recording of the transplanted RCS rats. (A) Individual traces. (B) Averaged traces. LE: left eye and RE: right eye.

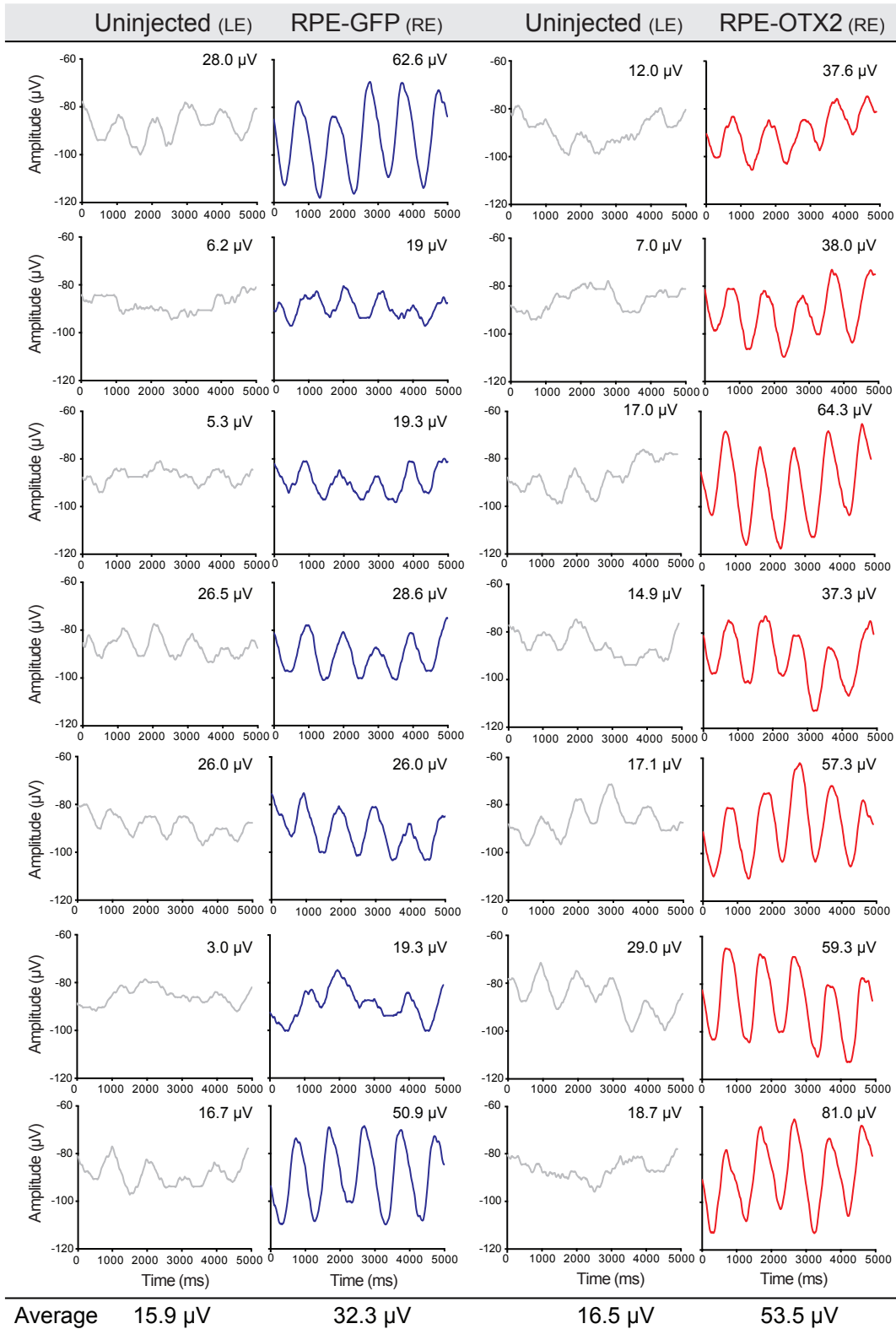
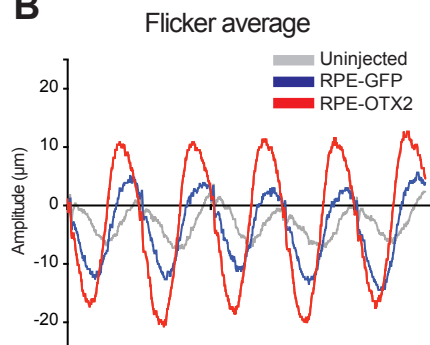
A**B**

Figure S5. Flicker ERG recording of the transplanted RCS rats. (A) Individual traces. (B) Averaged traces. LE: left eye and RE: right eye.

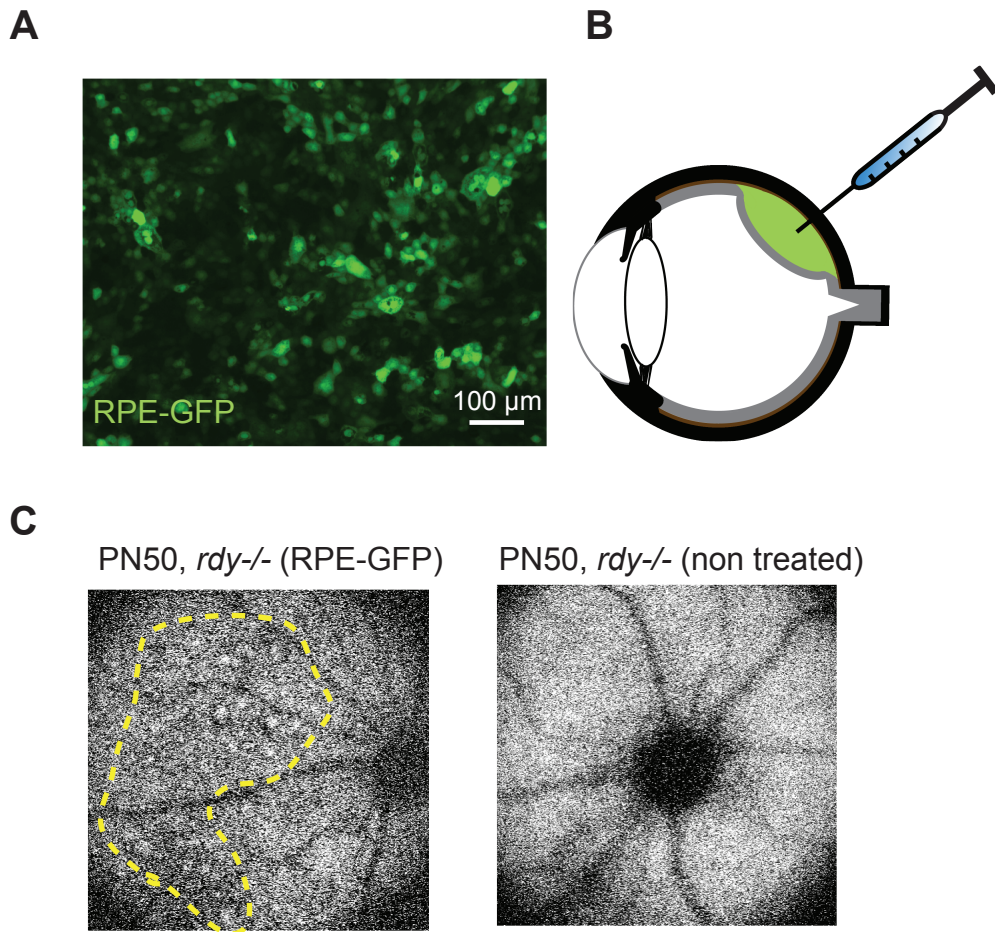


Figure S6. Transplantation procedure. (A) GFP fluorescence of the pig RPE cells transduced with AAV2.1-GFP prior to their transplantation. (B) Schematic representation of subretinal injection. (C) Representative fundus of the RPE-GFP graft validation in the eye of treated RCS rats and negative control (non-treated) using laser scanner ophthalmoscopy.

Table S1 Primer sequence used for quantitative RT-PCR

Gene Name	NCBI Accession No	Specie	PRIMERS (5' > 3')	
			Forward	Reverse
<i>ACTA2</i>	XM_005671254.1	Pig	GCCCTGGACTTTGAAAATGA	GTCAGGCAGCTCGTAGCTCT
<i>BDNF</i>	NM_214259.2	Pig	CCTTTGGAGCCTCCTCTTCT	CCTCATGGACATGTTTGACAG
<i>BEST1</i>	NM_001139443.1	Human	AAGACTGTGAGTTCTGGGGC	ACTCCACAGTTTTCCTCCTCAC
<i>BEST1</i>	XM_003353833.1	Pig	CCAAAGACCAGAGCCTTCAG	GACAGATCGGCCAGGTTAAA
<i>BMP4</i>	NM_001101031.2	Pig	GGCTGGAATGACTGGATTGT	AGTTGACCAGGGTCTGAACG
<i>CACNB2</i>	XM_003482816.1	Pig	CATGAACAGAGAGCCAAGCA	GGAGGTGTCGATTTTCTGGA
<i>CRX</i>	NM_000554.4	Human	GTGAGGAGGTGGCTCTGAAG	CTGCTGTTTCTGCTGCTGTC
<i>CRX</i>	XM_003127265.1	Pig	CTGCCTCTGCTTTCTGCTCT	GGATCCAGGCCACTGAAATA
<i>CLDN19</i>	NM_001160084.1	Pig	CATGGTCCTCAGTGTGTGCG	AGGGTGGCATACCATGAGAC
<i>CNTF</i>	XM_003122707.2	Pig	AACCTGGACTCTGTGGATGG	ACGGTAAGCTCGGAGGTTCT
<i>COL8A1</i>	XM_001926443.3	Pig	CCACCTCAAATTCCTCCTCA	CCGGCTGAATTCCTTCATA
<i>CRYAB</i>	XM_003357294.1	Pig	CGAGGAACTCAAGGTCAAGG	AGGACCCCATCAGATGACAG
<i>CTSD</i>	NM_001037721.1	Pig	ATCAAGGTCGAGAGGCAGAC	TCTGCTGCATCAGGTTATCG
<i>DCT</i>	NM_001025227.1	Pig	ACAGCGAGAAACTGCCAAGT	AAGGCATTCCGTAAGCTGAA
<i>FADS1</i>	NM_001113041.1	Pig	CTATGTGCGCATCTTCCTCA	CTGTGTCACCCACACAAACC
<i>GAPDH</i>	NM_001256799.1	Human	TCCAAAATCAAGTGGGGCGA	AAATGAGCCCCAGCCTTCTC
<i>GAPDH</i>	NM_001206359.1	Pig	ACCCAGAAGACTGTGGATGG	AAGCAGGGATGATGTTCTGG
<i>GPNUMB</i>	NM_001098584.1	Pig	GATTCATGATCCCAGCCACT	TTGCACAGTGAGGTTGAAGC
<i>ITGAV</i>	NM_001083932.1	Pig	GTTCTGCTTAAAGGCGGATG	GTCGAATTGCTCCTTTCTGC
<i>ITGB5</i>	NM_001246669.1	Pig	TGCAGACCAAGAGAGATTG	GTTTTGGCAGGTCTGGTTGT
<i>ITGB8</i>	NM_001097424.1	Pig	GCCTGGGTGTTTTTCAGTTGT	TTGAGCACACCATCCACATT
<i>KCNJ13</i>	NM_001172416.1	Human	GTGCTTTTGTGGCGAAGATT	GTGTTGGCCACTTGAAGAT
<i>KCNJ13</i>	XM_001926506.3	Pig	TGTCCACTGGCTTGTCTTTG	GGAGAAGGCAGCTGTGAAAC
<i>KRT8</i>	NM_001159615.1	Pig	GCATGAGTCTGGTTGGAGGT	AGCAGGCTCTGGTTCACTGT
<i>KRT18</i>	XM_003126180.3	Pig	ACCTCAGGACCTCAGCAAGA	GTGTCATCTCAGCAGCTCCA
<i>LHX2</i>	NM_001170519.1	Pig	AGTTCAGGCGCAACCTCTTA	GCAGGGTGGGACTAGTCAAG
<i>LRAT</i>	NM_001244920.1	Pig	AGTGTCTTGTCTTCGGCAGT	TTAGCCAGCCATCCATAAGC
<i>LRP8</i>	NM_001199891.1	Pig	AAGGAGTGCGAAGAGAACCA	TCACAGGTGAAGTCGCTGTC
<i>MERTK</i>	NM_006343.2	Human	TATTCGCGATTGGAGACAGG	GGGCAATATCCACCATGAAC
<i>MERTK</i>	XM_003124812.3	Pig	TCCTGGAAAAGTCTCCCTGAA	CCGGAAAAGTGTGTGTTGATG
<i>MITF</i>	NM_000248.3	Human	CTCGAGCTCATGGACTTTCC	TGATGATCCGATTCACCAA
<i>MITF</i>	NM_001038001.1	Pig	GCCAATCGGCATTTGTTACT	GGATGAGGGAAAAGTCCATGA
<i>MKI67</i>	NM_001101827.1	Pig	CCTCGAAAATCCAGAAAAGCA	GCCAAGGCCTCTTTCTTCTT
<i>OTX2</i>	NM_001270523.1	Human	CTTCTACTTTTGGGGGCATGGA	GCATTGGTACCCATGGGACTGA

			CTGTG	GTGTG
<i>OTX2</i>	XM_003353491.1	Pig	AGAGGTCCTACCCCATGACC	AGTAGGAAGTCGAGCCAGCA
<i>PMEL</i>	NM_001200053.1	Human	CTGGATGGTACAGCCACCTT	GGCACTTTCAATACCCTGGA
<i>PMEL</i>	XM_003481626.1	Pig	AACCCAATGCTACCTGCATC	CCAGGCCTTCCAGACATAAA
<i>RDH10</i>	NM_172037.4	Human	AAGGCTTTTCTTCCCTACGAT	GGCACAGTAATCCTCAACTC
<i>RDH10</i>	XM_001928082.3	Pig	AAGGCTTTTCTTCCCACCAT	GGCGCAGTAATCCTCAACTC
<i>RPE65</i>	NM_000329.2	Human	AAAGATCCCACCACTGCAAG	GATGGCTTGAATCGGTCACT
<i>RPE65</i>	XM_003127931.2	Pig	AGGCTGACACAGGCAAGAAT	AGCCAGATGGTCTCGTCACT
<i>SERPINF1</i>	NM_001078662.1	Pig	GCTTACTTCAAGGGGCAGTG	AGAATCCAAGCCGTAGCGTA
<i>SLC16A3</i>	XM_003357925.1	Pig	TACCTCACCCTGGGGTCAT	TAGCGGTTGAGCATGATGAG
<i>SLC16A8</i>	NM_013356.2	Human	CTGAGGCTGAAGGGGACTCT	GCCTCGCCTCTATTTCTGGT
<i>SLC16A8</i>	XM_003126028.1	Pig	CAAGGCAGTGAGCGTTTTCT	CCAGGCTGTGTCACTGTAGC
<i>SLC16A12</i>	NM_213606.3	Human	CCACACAACCTCCAGAGCAGA	GTCTGTGCCCATTTCTTTGGT
<i>SLC16A12</i>	XM_001928811.2	Pig	CTATGCACAGACAGCCTGGA	TGACAACACTCCCAAGTGGA
<i>SLC24A5</i>	XM_003121523.1	Pig	TCTCCATCATCGGAGTTTCC	CAAGGCCATGAGCATGTAGA
<i>SLC39A12</i>	XM_003130728.1	Pig	ACGGAAAACCCCTCAACTCT	TTGCCTTCCATACGTTCTCC
<i>SMAD6</i>	XM_003480446.1	Pig	CACCCCATCTTCGTCAACTC	CGAAGTCGAACACCTTGATG
<i>TRPM3</i>	XM_001925032.3	Pig	AGGAGACCTTGTCCCAACT	TGCAGGAGCTTTGGATTCTT
<i>TYR</i>	NM_000372.4	Human	CCATGGATAAAGCTGCCAAT	ATTGTGCATGCTGCTTTGAG
<i>TYR</i>	NM_001025212.1	Pig	ATGGAACCATGTCCCAGGTA	CATTGGCTTCCGGATACACT
<i>TYRP1</i>	NM_001025226.1	Pig	TGTCTGATTCGTGCCAGAAG	GTGAGGAGAGGCTGATTTGC
<i>VIM</i>	XM_005668106.1	Pig	ATGCTTCTTTGGCACGTCTT	CTCATCAGGGCTCCAGCTAC