

Supplementary Materials

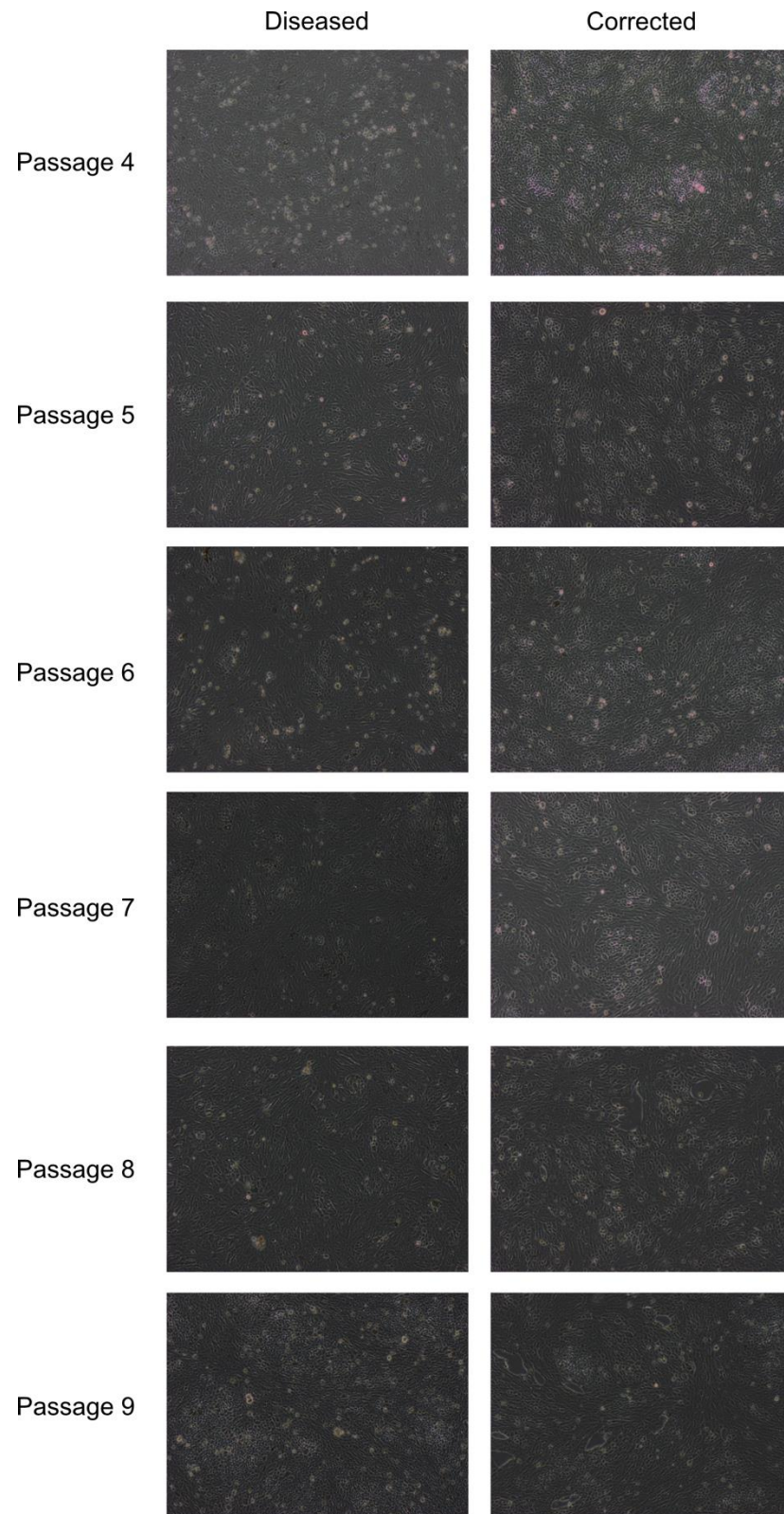


Figure S1. Atrophy of RPE upon extended passage, maintenance of polygonal morphology (10× objective).

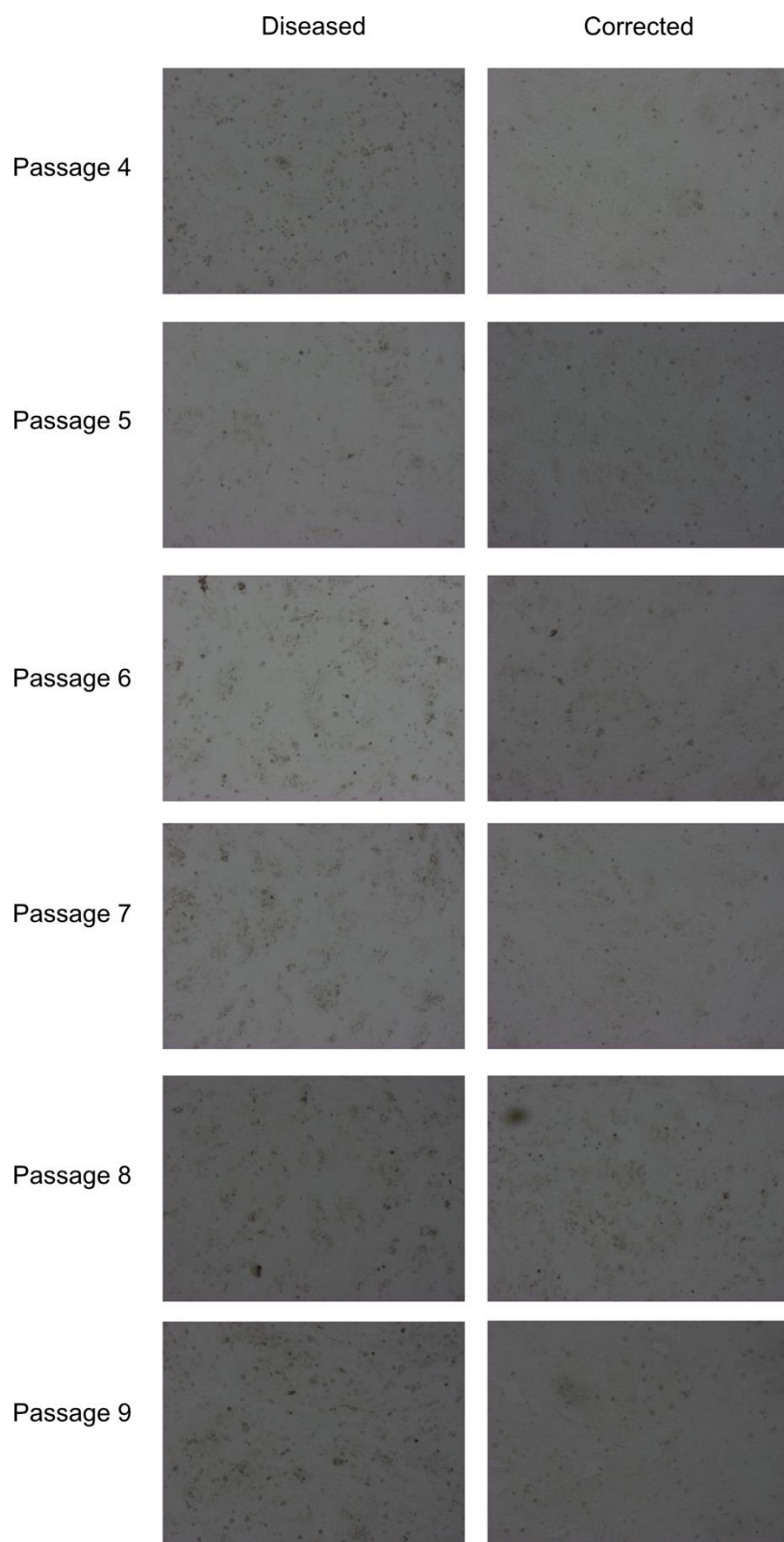


Figure S2. Atrophy of RPE upon extended passage, maintenance of pigmentation (10× objective).

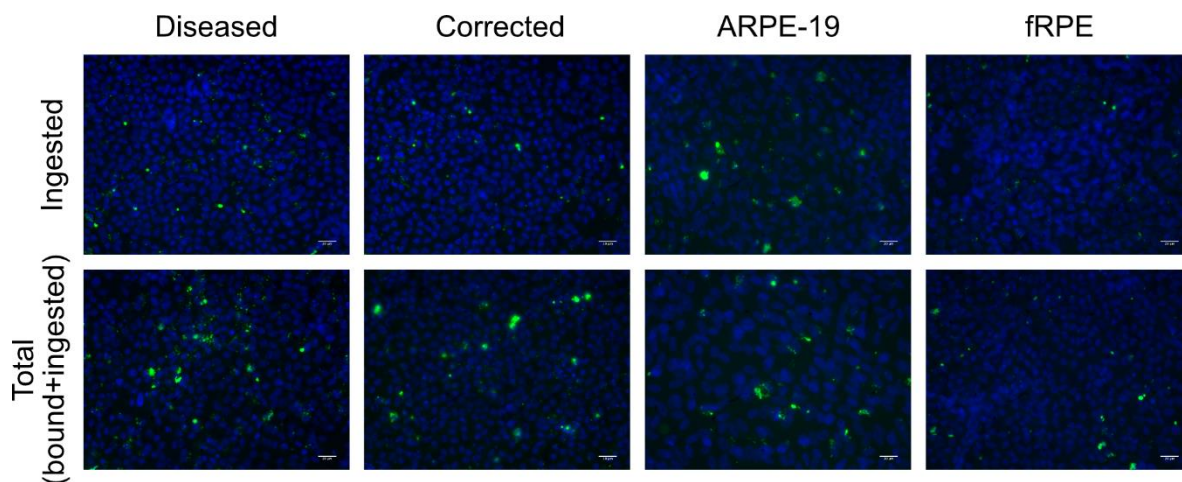


Figure S3. Representative images of bound and ingested FITC-labeled POS in RPE (10 $\times$ objective). Scale bar equals 20 μ m.

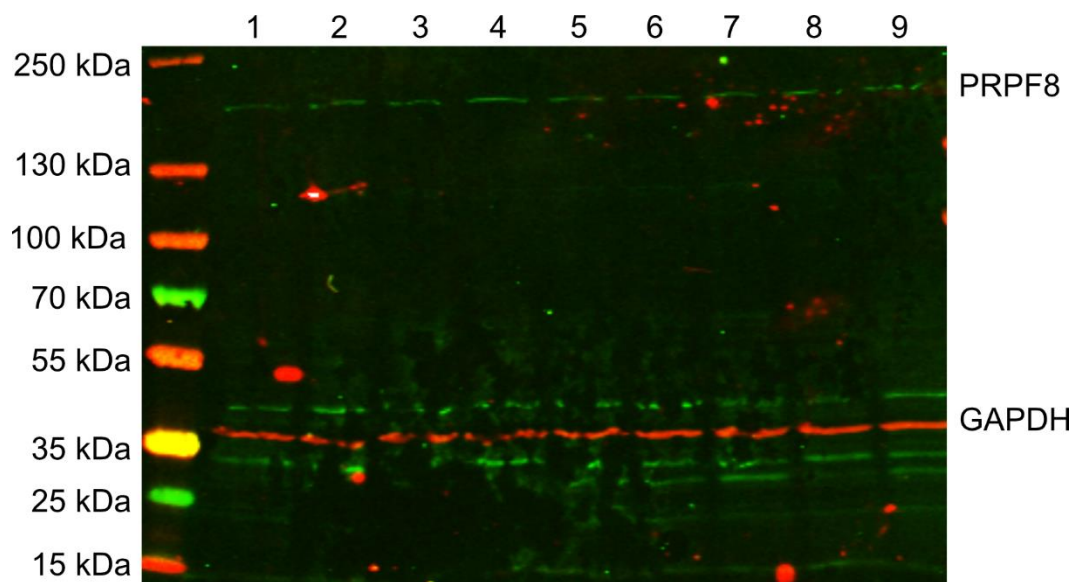
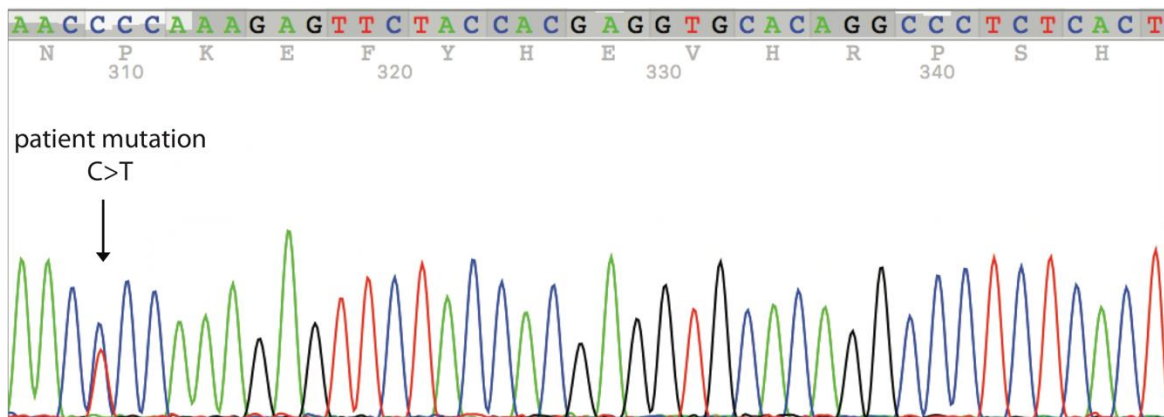


Figure S4. Western blot detection of PRPF8 and GAPDH loading control from passage 3 day 30 stem cell-derived RPE. (1–3) diseased clones, (4–6) corrected clones, (7–9) wild-type H9, UCSF4, and MyCell, respectively. 10 μ g of protein per lane.

Uncorrected



Gene corrected (homozygous)

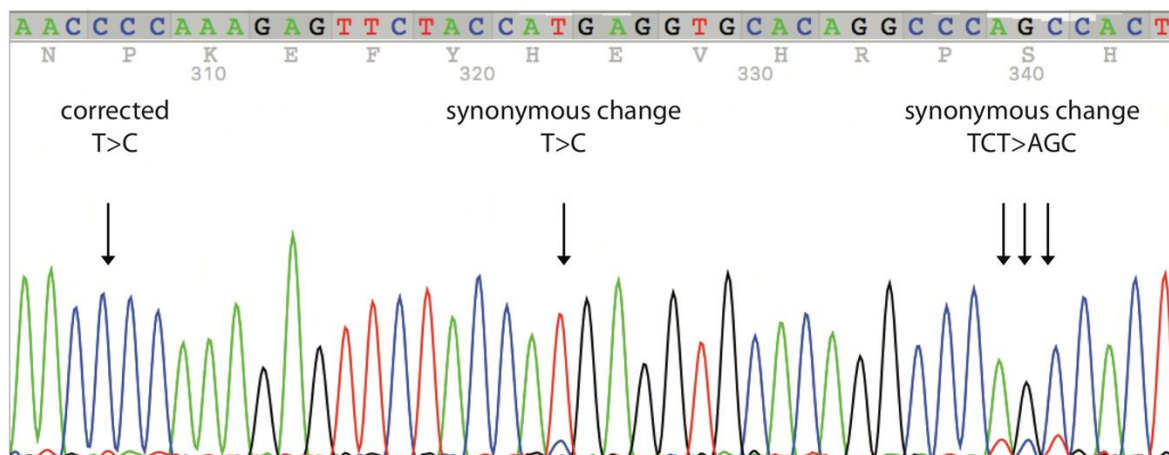


Figure S5. Confirmation of *PRPF8* gene-correction. mRNA encoding Cas9-Gem and a ssODN repair template was introduced into patient-specific iPSC via electroporation.

Table S1. Primary Antibodies.

Target Protein	Clonality/Immunoglobulin	Host	Dilution	Source/ Catalog Number
PMEL17	Monoclonal	Mouse	1:100	Dako/M0634
PRPF8	Polyclonal	Rabbit	1:100	abcam/ab87433, ab79237
ZO-1	Polyclonal	Rabbit	1:100	abcam/ab59720
BEST1	Monoclonal/Igg1	Mouse	1:100	abcam/ab2182
GAPDH	Monoclonal/Igg1	Mouse	1:100	ThermoFisher/MA5-15738

Table S2. Secondary Antibodies.

Target	Fluorophore/Immunoglobulin	Host	Dilution	Source/Catalog Number
Anti-rabbit	Cy2/IgG (H+L)	Donkey	1:300	Jackson Immuno/711-225-152
Anti-mouse	Cy3	Donkey	1:300	Jackson Immuno/715-165-150
Anti-rabbit	Cy3	Donkey	1:300	Jackson Immuno/711-165-752
Anti-mouse	800CW	Donkey	1:10,000	LI-COR/925-32212
Anti-rabbit	680RD	Donkey	1:10,000	LI-COR/925-68073

Table S3. Taqman Probes.

Target	Assay ID Number
RPE-specific protein 65 kDa (<i>RPE65</i>)	Hs01071462_m1
Bestrophin 1 (<i>BEST1</i>)	Hs00188249_m1
Retinaldehyde binding protein 1 (<i>RLBP1</i>)	Hs00165632_m1
Microphthalmia-associated transcription factor (<i>MITF</i>) isoform 2	AJD1S3G
Tyrosinase (<i>TYR</i>)	Hs00165976_m1
Paired box 6 (<i>PAX6</i>)	Hs01088112_m1
Pan microphthalmia-associated transcription factor (<i>MITF</i>)	Hs01117293_m1
Zinc finger protein 42 (<i>REX1</i>)	Hs01124465_m1
Splat-like transcription factor 4 (<i>SALL4</i>)	Hs00360675_m1
S100 calcium binding protein A4 (<i>S100A4</i>)	Hs00243202_m1
Integrin, α 2 (<i>ITGA2</i>)	Hs00158127_m1
Microphthalmia-associated transcription factor (<i>MITF</i>) isoform 4+5	Hs01117294_m1
Platelet/endothelial cell adhesion molecule 1 (<i>PECAM1</i>)	Hs00169777_m1
Microtubule-associated protein 2 (<i>MAP2</i>)	Hs00258900_m1
Marker of proliferation Ki-67 (<i>MKI67</i>)	Hs01032443_m1
Tyrosinase-related protein 1 (<i>TYRP1</i>)	Hs00167051_m1
Premelanosome (<i>PMEL</i>)	Hs00173854_m1
Eukaryotic translation initiation factor 2B, subunit 2 β (<i>EIF2B2</i>)	Hs00204540_m1
Ubiquitin-conjugating enzyme E2R 2 (<i>UBE2R2</i>)	Hs00215107_m1
Small EDRK-rich factor 2 (<i>SERF2</i>)	Hs00428481_m1