

**OMTM, Volume 15**

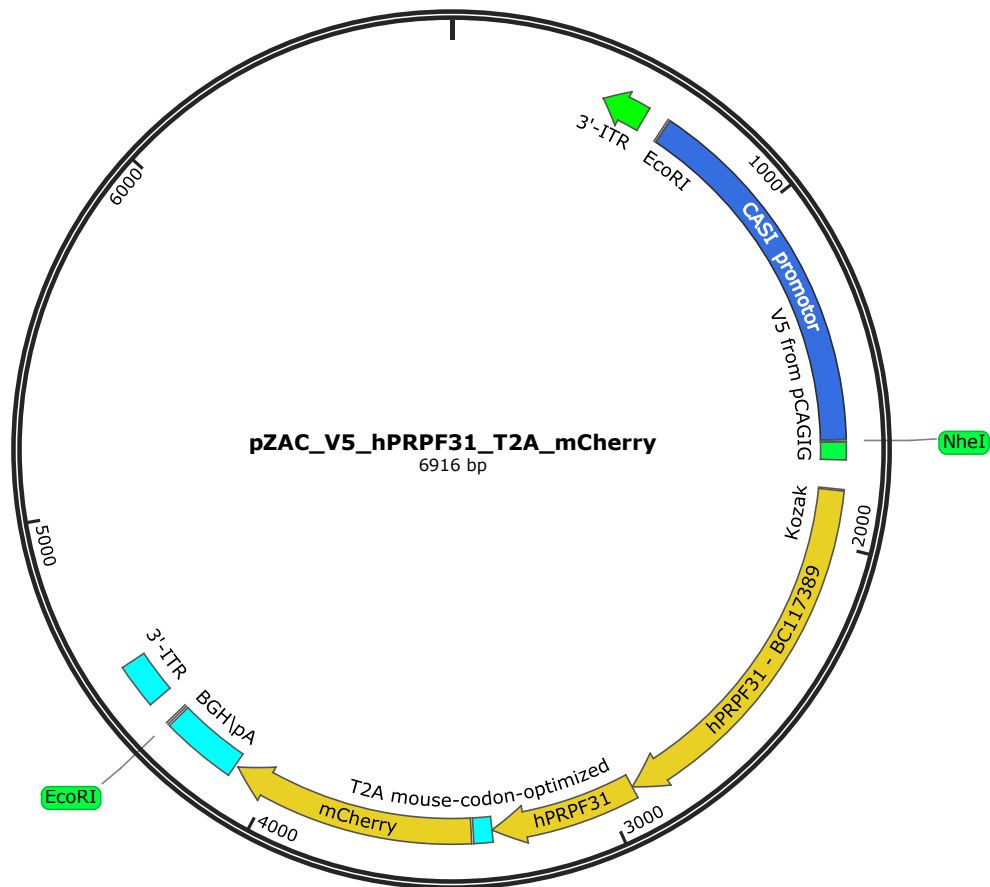
**Supplemental Information**

**AAV-Mediated Gene Augmentation**

**Therapy Restores Critical Functions**

**in Mutant PRPF31<sup>+/-</sup> iPSC-Derived RPE Cells**

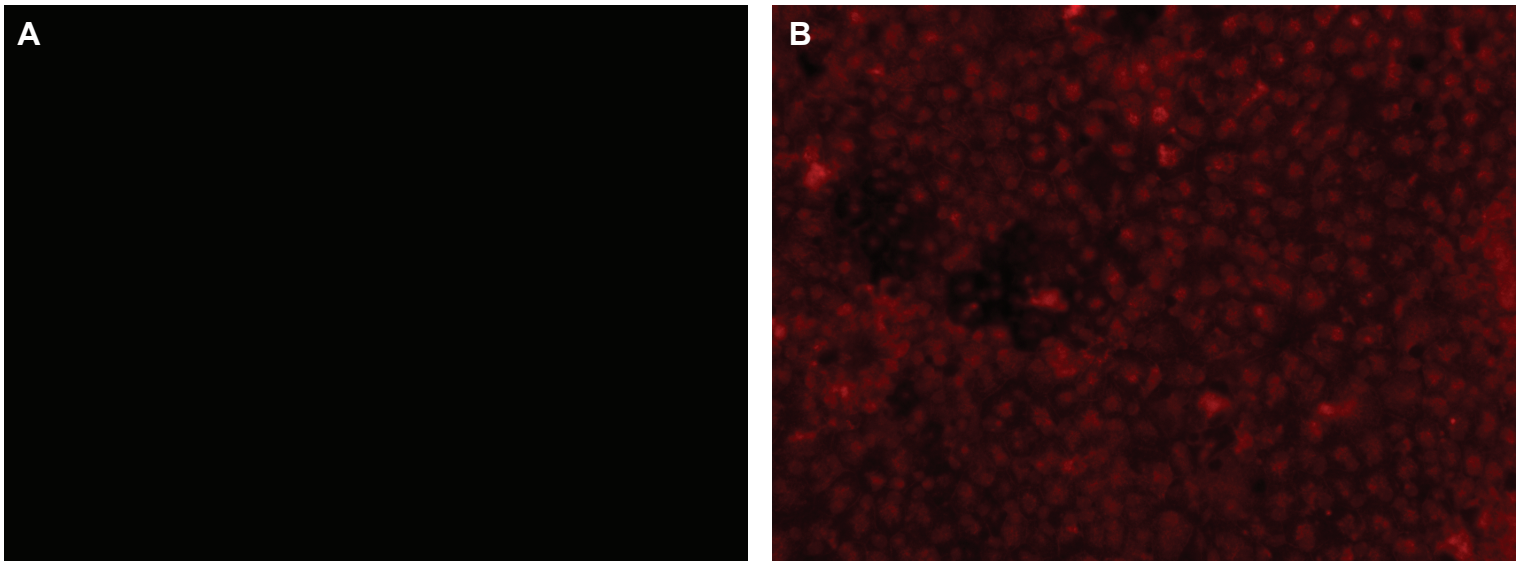
**Elizabeth M. Brydon, Revital Bronstein, Adriana Buskin, Majlinda Lako, Eric A. Pierce, and Rosario Fernandez-Godino**



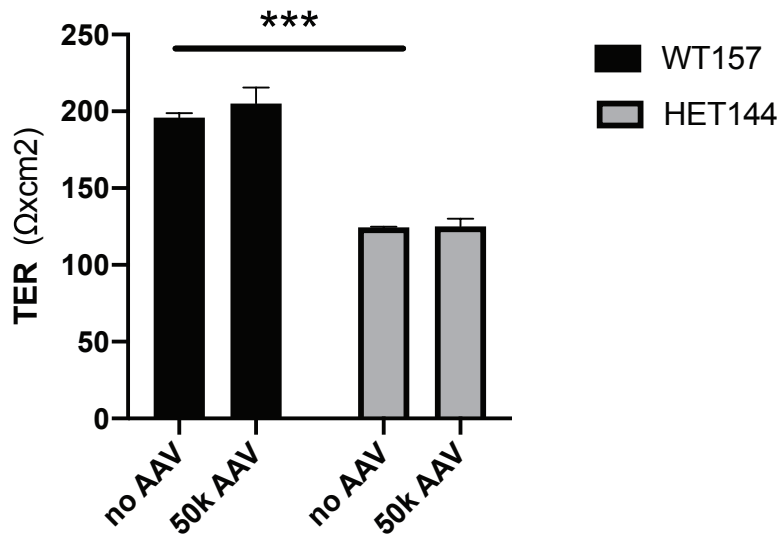
**Figure S1.** Map of the AAV vector used for AAV-mediated expression of PRPF31 in iPSC-RPE cells.

iPSC-RPE *PRPF31*<sup>+/-</sup> no AAV

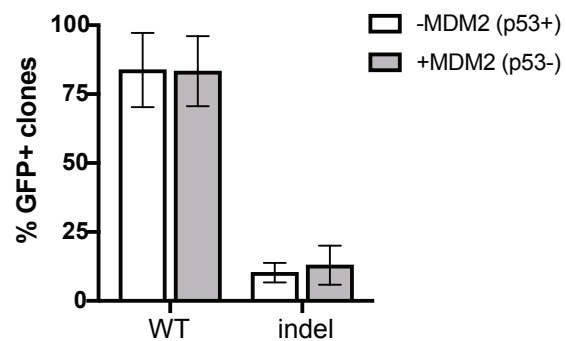
iPSC-RPE *PRPF31*<sup>+/-</sup> +AAV-mCherry



C



**Figure S2.** (A) iPSC-RPE control and (B) treated with 50,000 GC/cell of AAV-*PRPF31* imaged with confocal. AAV-derived mCherry fluorescent signal shows that AAV targeted most of the cells. (C) TER of iPSC-RPE *PRPF31*<sup>+/+</sup> and *PRPF31*<sup>+/-</sup> cells on transwells with and without treatment with AAV-*PRPF31*. (n=4/type. 2-way ANOVA. \*\*\*p<0.001. Data represented as mean +/- SD).



**Figure S3.** Percentage of GFP positive iPSCs with and without indels in PRPF31 after transfection with the plasmid containing Cas9 and the gRNA 1 in the absence (white bars) or presence (grey bars) of the p53 antagonist MDM2 (2-way ANOVA. Data represented as mean  $\pm$  SD).