Description of Additional Supplementary Files

File Name: Supplementary Data 1

Description: Summary of clinical data for all RP11 patients and controls.

File Name: Supplementary Data 2

Description: Differential gene expression in fibroblasts, iPSCs, retinal organoids and RPE

samples of all RP11 patients versus all WT controls (padj< 0.05).

File Name: Supplementary Data 3

Description: Summary of GO terms for cellular components of genes differentially expressed in fibroblasts, iPSCs, retinal organoids and RPE samples of all RP11 patients versus all WT controls

File Name: Supplementary Data 4

Description: Differential exon usage in fibroblasts, iPSCs, retinal organoids and RPE samples of all RP11 patients versus all WT controls (p< 0.05 and inclusion difference of > than 0.05).

File Name: Supplementary Data 5

Description: Summary of GO terms for cellular components of genes differentially spliced in fibroblasts, iPSCs, retinal organoids and RPE samples of all RP11 patients versus all WT controls

File Name: Supplementary Data 6

Description: A summary of sequencing data, antibodies, oligonucleotides, snRNP probes and CRISPR/Cas9 reagents.

File Name: Supplementary Data 7

Description: Off-target site sequencing analysis for gRNA (AGATCCAACCGTATGAGCTTCGG)

of PCR products using corrected RP11-1VS patient genomic DNA.

File Name: Supplementary Data 8

Description: Differential protein abundance between RP11VS and Cas9-RP11VS corrected

RPE cells and associated GO analysis.

File Name: Supplementary Data 9

Description: Differential protein abundance between RP11VS and Cas9-RP11VS corrected

retinal organoids and associated GO analysis.