ID No.	K2013
Project Title	Establishment of a platform for development and evaluation of exon skipping based gene therapy tool by using human iPS cell
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Report

The exon skipping vector targeting to the EYS mutation was designed and constructed and examined its effects using hiPSC derived RPE.
Allele specific exon skipping by using SNP was performed in hiPSC derived RPE and the vector worked well to act on allele specific manner.
Technique for nucleotide substitution by using CRISPR/Cas9 vector had been introduced to IMSUT, and vectors targeting genes related to photoreceptor degeneration by their mutations were constructed. Biochemical and functional assay systems to examine RPE functions were established, and effects of shRNA to RPE were examined by newly established assay system.