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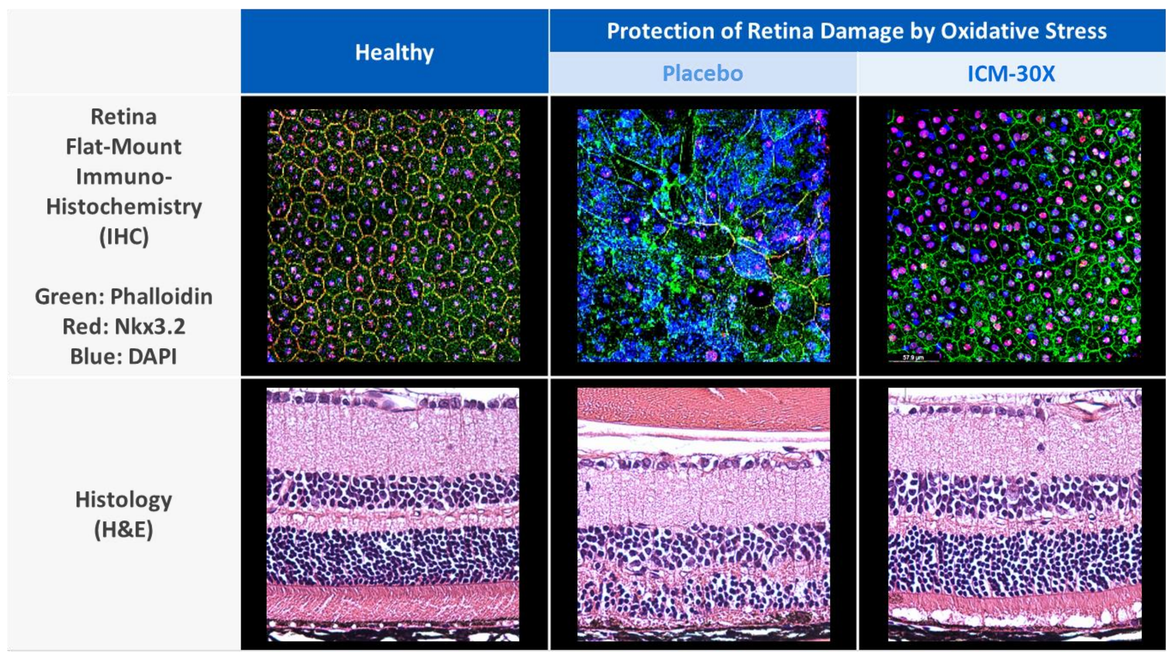
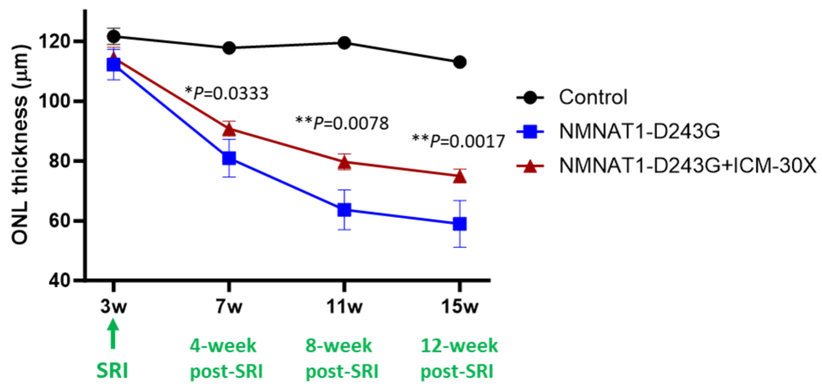
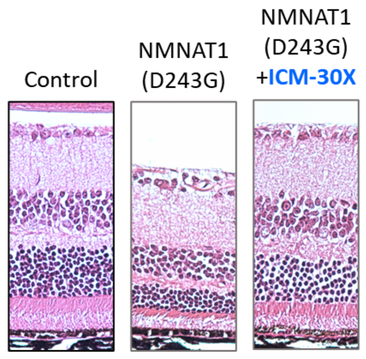
AAV Gene Therapy for Degenerative Retinal Diseases

ICM CO., LTD.

Disease Area	***Ophthalmology***
Product Type	Recombinant Adeno-Associated Virus (rAAV)
Indication	Dry-AMD and Inherited Retinal Diseases (IRDs)
Target	Nkx3 Homeobox 2 (Nkx3-2)
Mechanism of Action	• Supporting RPE viability by suppressing RIP3-mediated RPE necroptosis. • Suppressing inflammatory responses by inhibiting inflammatory cytokine and chemokines. • Inhibiting blood vessel invasion by inducing lysosomal protein degradation of HIF-1α.
Competitiveness	• A novel gene therapy for retinal degeneration with a validated target, apart from the limited treatments employing VEGF inhibition approach. • Various biological activities of Nkx3.2 can control molecular events associated with retinal degeneration including a broad range of IRDs. • First-in-class AAV-based gene therapeutics has been developed and verified to be effective and safe. • Patient convenience and benefits from durable efficacies by single injection.
Development Stage	***Candidate***
Route of Administration	Subretinal injection (SRI)
Key Data	**A**  ☑ Retina damage was induced by intravenous injection (IVI) of sodium iodate (20 mg/kg) ☑ Histological analyses demonstrate that oxidative stress disrupts overall retinal structure, and sub-retinal injection of ICM-30X protects these structural damages. **B** Longitudinal Measurement of Retina Thickness (OCT)  A significance compared to non-treated NMNAT1-D243G by two-way ANOVA. Histological assessment (H&E staining)  ☑ Retinal degeneration was observed in NMNAT1-D243G Mice. ☑ Longitudinal in-life OCT assessment and histological analyses demonstrate that sub-retinal injection of ICM-30X can protect structural damages caused by NMNAT1 gene mutation.