RS-2024-00344469

AAV Gene Therapy for Degenerative Retinal Diseases

ICM CO., LTD.

OPHTHALMOLOGY | **Candidate**

Product Type	Recombinant Adeno-Associated Virus (rAAV)
Indication	Dry-AMD and Inherited Retinal Diseases (IRDs)
Target	Nkx3 Homeobox 2 (Nkx3-2)
MoA(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)

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