RS-2024-00339507

Development of Retina-Specific AAV and Gene-Editing Therapeutics for Leber Congenital Amaurosis type 10 based on the TaRGET System

GenKOre

OPHTHALMOLOGY | Lead

Product Type	Engineered Adeno-associated virus harboring TaRGET system (AAV-TaRGET-gRNA1/2)
Indication	Leber Congenital Amaurosis type 10 (LCA10) disease
Target	CEP290 gene (IVS26 mutation)
MoA(Mechanism of Action)	• Deletion of the IVS26 mutation in the genome • Induction of the expression of the normal CEP290 protein • Increase in function of cone and rod cells
Competitiveness	• Size: The hypercompact size of the TaRGET system offers the advantage of compatibility with delivery via AAV, which has a restricted payload size of approximately 4.7 kb. • Retina specific engineered AAV: Engineered AAVs for photoreceptor-specific delivery can significantly reduce the required dosage, thereby improving safety (immune responses and side effects)and dramatically improving the efficacy of the therapeutics.
Development Stage	Lead optimization
Route of Administration	• Subretinal injection(SR)

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