HN22C0300

Non-clinical development of gene therapy for Retinitis Pigmentosa with an RNA repair mechanism using AAV vector that encodes trans-splicing ribozyme targeting rhodopsin RNA

Rznomics inc.

Rznomics

OPHTHALMOLOGY | **Non-Clinical**

Product Type	Trans-Splicing Ribozyme (RNA replacement)
Indication	Autosomal dominant Retinitis pigmentosa (adRP)
Target	Rhodopsin mutation RNA
MoA(Mechanism of Action)	Cleavage of mutant rhodopsin (RHO) mRNA and substitution with normal RHO mRNA through RNA replacement enzyme → Expression of normal RHO protein specifically in photoreceptor cells.
Competitiveness	First in class • RNA editing: ribozyme-mediated gene therapy • Act w/ its own mechanism (No external proteins) • Induction of desired RNA expression at target RNA • Capable of targeting variable mutation sites
Development Stage	Non-Clinical
Route of Administration	Sub-retinal injection

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