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Development of new compounds for effective gene editing therapy for an IMPDH1 mutation causing autosomal dominant retinitis pigmentosa

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OPHTHALMOLOGY | **Hit**

Product Type	Gene editing therapy
Indication	Retinitis pigmentosa
Target	IMPDH1; Autosomal dominant retinitis pigmentosa; adRP; gene editing therapy
MoA(Mechanism of Action)	CRISPR-Cas9-mediated gene editing effectively and safely destroying the IMPDH1-R316P mutant allele inducing the autosomal dominant retinitis pigmentosa in the retina.
Competitiveness	1. As an ophthalmologist with extensive experience, I can ensure that all steps of development and evaluation are carried out under clinically relevant conditions. 2. We have already established essential components. - The patient-derived induced pluripotent stem cells (iPSC) - The patient-derived sequence-bearing mouse model recapitulating the disease. - Ultra-Precision gene editing strategy distinguishing 1-bp mismatches. - A novel AAV vector system expressing Cas9 and a guide RNA from a single vector.
Development Stage	Hit
Route of Administration	Subretinal injection

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