

Lead generation of prime editor for genetic variants of Leber congenital amaurosis

Seoul National University



서울대학교
SEOUL NATIONAL UNIVERSITY

OPHTHALMOLOGY	Hit
Product Type	Adeno-associated virus containing prime editors
Indication	Leber congenital amaurosis (LCA) type 10
Target	Genetic variants in the CEP290 gene
MoA(Mechanism of Action)	CRISPR-based primed editing of genetic variants in the CEP290 gene
Competitiveness	Complete correction of genetic variants in the exons <ul style="list-style-type: none"> • Antisense oligonucleotides: mainly targeting intron variants • Gene delivery: no correction of genetic variants
Development Stage	Hit
Route of Administration	Subretinal injection