

Development of AI platform-based AAV engineering and gene therapy for choroideremia

Aavatar Therapeutics



OPHTHALMOLOGY		Hit
Product Type	Gene & Nucleic acids	
Indication	Choroideremia	
Target	CHM gene	
MoA(Mechanism of Action)	AAV-mediated gene augmentation therapy	
Competitiveness	• The previous AAV2-REP1 gene therapy (timrepigene emparvovec, Biogen) failed clinically due to the intrinsic limitations of the wild-type AAV2 capsid. • There are currently no active or newly initiated clinical trials of AAV-based therapies for choroideremia	
Development Stage	Hit	
Route of Administration	Intravitreal injection	

Any unauthorized distribution or reproduction of this material is strictly prohibited.