RS-2023-00259174

Development of gene-editing therapeutics for Huntington's disease with CAG repeat expansion

GenKOre

NEUROSCIENCE | Hit

Product Type	Adeno-associated virus (AAV) harboring Target system (Cas12f1-gRNAs)
Indication	Huntington's disease
Target	HTT gene (CAG repeat expansion)
MoA(Mechanism of Action)	One-time administration of the AAV-delivered gene editors results in the deletion of the expanded CAG repeats, thereby decreasing the accumulation of the toxic polyQ peptides, RNA foci, and other related pathological events. The cleaved site is recovered into wild-type sequence through homology-directed repair.
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. • One-time gene-editing therapy: Some pharmaceutical companies have developed a synthetic antisense oligonucleotides (ASOs)-based therapy to target the expanded CAG repeats of the HTT gene. Given the relatively short treatment duration of ASO therapy, it necessitates the periodic administration of the treatment to the brain, which can impose a burden on patients. In contrast, gene-editing therapeutics have the potential to permanently edit the mutant genome itself, offering a more radical and competitive therapeutic option.
Development Stage	Hit
Route of Administration	• Intrastriatal (ISTR) injection • Intracerebroventricular (ICV) injection

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