

METHODS AND COMPOSITIONS FOR THE TREATMENT OF HUNTINGTON'S DISEASE

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PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20260055400	02/26/2026	2022-144
European Patent Office	Published Application	4544046	04/30/2025	2022-144

BRIEF DESCRIPTION

There are no approved disease-modifying therapies for Huntington's disease (HD), a fatal neurodegenerative condition caused by a heterozygous expansion of a CAG array in exon 1 of Huntingtin (Htt). Typically, HD patients are heterozygous for the toxic gain of function disease allele, yet expression of the wildtype version of the gene is essential.

The inventors have developed methods and compositions to selectively silence expression from the disease-associated allele while leaving the wildtype version intact. The invention relies on the introduction of a 'poison' exon into the diseased allele wherein introduction of the poison exon may be accomplished by standard methods in the art, such as introduction of the exon sequences through homology-directed repair following targeted nuclease cleavage, transposon-associated targeted sequence introduction, base editing, and prime editing. Following the introduction of the poison exon, post-transcriptional splicing results in an RNA that is susceptible to nonsense mediated decay due to the introduction of a stop codon in the introduced exon. RNAs comprising the poison exon are subsequently degraded in the cell, effectively silencing expression of the mutant disease-associated allele.

SUGGESTED USES

Novel therapeutic for the treatment and/or prevention of Huntington's Disease.

ADVANTAGES

RELATED MATERIALS

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [COMPOSITIONS AND METHODS FOR REDUCING RNA LEVELS](#)

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OTHER INFORMATION

KEYWORDS

therapeutic, Huntington's Disease, neurodegenerative

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