

# METHODS AND COMPOSITIONS FOR THE TREATMENT OF HUNTINGTON'S DISEASE

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## PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	WO 2023/250325	12/28/2023	2022-144

Additional Patent Pending

## 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

### CATEGORIZED AS

- » [Biotechnology](#)
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### RELATED CASES

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