METHODS AND COMPOSITIONS FOR THE TREATMENT OF HUNTINGTON'S DISEASE

Tech ID: 32861 / UC Case 2022-144-0

PATENT STATUS
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

INVENTORS
» Lareau, Liana F.

OTHER INFORMATION
KEYWORDS
therapeutic, Huntington's Disease, neurodegenerative

CATEGORIZED AS
» Biotechnology
» Genomics
» Health
» Medical
» Gene Therapy
» Therapeutics

RELATED CASES
2022-144-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS
» COMPOSITIONS AND METHODS FOR REDUCING RNA LEVELS