ALLELE SELECTIVE EPIEDITING OF HUNTINGTON'S DISEASE LOCUS

Tech ID: 33485 / UC Case 2024-084-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Huntington's disease is an autosomal dominant neurodegenerative disease that has a wide impact on a person's functional abilities and usually results in movement, cognitive, and psychiatric disorders. A treatment for HD that can selectively reduce expression of a mutant copy of the HTT gene (e.g., while having little to no effect on expression of a normal copy of the HTT gene) is highly desirable.

UC Berkeley researchers have discovered methods and compositions that take advantage of naturally occurring single nucleotide polymorphisms (SNPs) in the regulatory region of the HTT gene to distinguish between an HTT gene allele containing a pathogenic CAG trinucleotide repeat expansion and an HTT allele that lacks a pathogenic CAG trinucleotide repeat. Potential benefits of using this strategy include 1) lowering both mutant mRNA and mutant protein levels while normal allele remains unaffected, 2) lowering levels of toxic HTTexon11 species and 3) by lowering transcription across the mutant CAG repeats could slow down repeat expansion in mutant allele poten5ally leading to a delay in age of onset.

SUGGESTED USES

» Therapeutic treatment of Huntington's disease

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INVENTORS

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OTHER INFORMATION

CATEGORIZED AS

» **Biotechnology**

» Health

» **Medical**

» Disease: Central Nervous System

» Therapeutics

RELATED CASES

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