METHOD FOR ALLELE SELECTIVE EXCISION OF HUNTINGTIN GENE USING CRISPR EDITING

Tech ID: 33954 / UC Case 2025-098-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Huntingtin disease (HD) is a heritable neurodegenerative disorder affecting up to 1 in 10,000 people, with an average survival duration of 17-20 years post symptom onset. HD patients typically suffer from severe motor/coordination decline and weight loss. There is no cure for HD, and traditional small molecule drugs only address symptom management. Prior approaches to treatment have failed for several reasons. Protein-targeting approaches such as ubiquitin ligase lack specificity, degrading both mutant and wild type huntingtin protein indiscriminately. Other approaches such as antisense oligonucleotides (ASOs) can target mutant RNA but require many doses over the patient's lifetime. The disorder affects the huntingtin gene (HTT), which is essential in transcription, reactive oxygen species detection, DNA damage repair, and axonal transport. HD is caused by a heterozygous polyglutamine repeat expansion in exon 1 of HTT. Genome editing is an attractive alternative therapy for HD, as it would require a single dose and is permanent.

UC Berkeley researchers have developed a system for CRISPR-based genome editing for genetic diseases like HD. Allele specific excision is possible through two different mechanisms: heterozygous SNPs that create/remove a PAM site, and heterozygous SNPs that create a mismatch within the seed region. For patients with these genotypes, the invention allows selective excision of the pathogenic repeats from only that allele. Over 20% of HD patients can be treated with just one of our novel candidate pairs, and about half of all patients could benefit from one of our novel candidate pairs.

SUGGESTED USES

» Gene editing therapy for Huntingtin disease

ADVANTAGES

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Methods For Selectively Disabling Oncogenes
- High-Yield Production Of Base Editor Enzymes Via Conjugation
- Nuclear Localization Signals Inside Cas9 To Enhance Genome Editing

CONTACT

Terri Sale
terri.sale@berkeley.edu
tel: 510-643-4219.

INVENTORS

» Wilson, Ross C.

OTHER INFORMATION

CATEGORIZED AS

» **Biotechnology**

» Genomics

» **Medical**

» Disease: Central Nervous System

» Gene Therapy

» Therapeutics

RELATED CASES

2025-098-0

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