

CRISPR-MEDIATED TRANSCRIPTIONAL AND SPLICING REGULATION OF ALPHA-SYNUCLEIN FOR THE TREATMENT OF NEURODEGENERATIVE DISORDERS

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BRIEF DESCRIPTION

Parkinson's disease and related synucleinopathies currently lack disease-modifying cures, as existing treatments only manage symptoms associated with alpha-synuclein (aSyn) aggregation. To address this, UC Berkeley researchers have developed a suite of non-viral, CRISPR-mediated strategies designed for the permanent downregulation of the endogenous SNCA gene and the LRRK2 risk allele. By utilizing precise base editing of regulatory regions, genomic excision of non-coding sequences, and splicing modulation, this platform provides a durable, single-administration alternative to transient RNA-targeting therapies. The invention further enables allele-selective knockout of the LRRK2 G2019S mutation, employing a specialized CRISPR-Cas9 variant to discriminate between mutant and wild-type genes, thereby offering a highly specific and permanent therapeutic intervention for neurodegenerative disorders.

SUGGESTED USES

- » THERAPEUTIC TREATMENT FOR PARKINSON'S DISEASE (IDIOPATHIC AND FAMILIAL)
- » INTERVENTION FOR OTHER ALPHA-SYNUCLEINOPATHIES, INCLUDING MULTIPLE SYSTEMATROPHY (MSA) AND DEMENTIA WITH LEWY BODIES
- » PROPHYLACTIC TREATMENT FOR INDIVIDUALS WITH GENETIC BIOMARKERS INDICATING HIGH RISK FOR PARKINSON'S

ADVANTAGES

- » Induces a durable genetic change that eliminates the need for repeated dosing associated with RNA-targeting therapies
- » Base editing avoids double-stranded breaks and unpredictable insertions/deletions (indels)
- » Capable of targeting specific risk mutations without impacting healthy wild-type alleles
- » Compatible with diverse delivery mechanisms including ribonucleoproteins (RNPs), lipid nanoparticles (LNPs), and peptide-mediated delivery

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [High-Yield Production Of Base Editor Enzymes Via Conjugation](#)
- ▶ [High-Performance CRISPR-Cas9 Engineered for Superior Therapeutic Gene Editing](#)

CONTACT

Ellen S. Kats
ellenkats@berkeley.edu
tel: .



INVENTORS

» Wilson, Ross C.

OTHER INFORMATION

CATEGORIZED AS

- » **Biotechnology**
- » Genomics
- » **Medical**
- » Gene Therapy
- » Research Tools
- » Therapeutics
- » **Research Tools**
- » Nucleic Acids/DNA/RNA

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