

Epigenetic Prevention and Treatment of CDKL5 Deficiency Disorder

Tech ID: 32876 / UC Case 2019-800-0

ABSTRACT

Researchers at the University of California, Davis have developed a targeted epigenetic approach for the prevention and treatment CDKL5 deficiency disorder.

FULL DESCRIPTION

CDKL5 is a gene found on the X-chromosome that is responsible for producing an essential protein for brain development and function. Mutations of this gene can lead to CDKL5 deficiency disorder (CDD), affecting around 1 in 40,000 individuals. Symptoms typically begin in infancy and include seizures, limited motor functionality, and developmental delays. Anti-seizure medications are used to treat symptoms of the disorder, but there are currently no comprehensive treatments for CDD. Research has been conducted on small-molecule drugs to treat CDD and similar X-linked disorders through gene reactivation; however, such drugs typically cause global gene reactivation of the X-chromosome, making them unsuitable for targeted treatments. Furthermore, these treatments have limited therapeutic potential as they rely on cell proliferation to function.

Researchers at the University of California, Davis have developed a new targeted approach for preventing and treating CDD. In particular, the new approach uses CRISPR/Cas9-based techniques to guide epigenetic modifiers to desired genomic loci, thus enabling a synthetic escape of the CDKL5 gene from X-chromosomal inactivation while avoiding global X-chromosomal reactivation.

APPLICATIONS

- Prevention or treatment of CDKL5 deficiency disorder

FEATURES/BENEFITS

- Focuses on target allele without global X-chromosome reactivation
- Does not rely on cell proliferation

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20220389393	12/08/2022	2019-800

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OTHER INFORMATION

KEYWORDS

X-chromosome

reactivation, XCR, CDKL5,

CDKL5 deficiency disorder,

CDD, X-chromosome

inactivation, XCI,

epigenetic, CRISPR

CATEGORIZED AS

- **Biotechnology**
 - Genomics
 - Health
 - Other
- **Medical**
 - Disease: Genetic Diseases and Dysmorphic Syndromes
 - Gene Therapy
 - Therapeutics
- **Research Tools**
 - Expression System
 - Nucleic Acids/DNA/RNA

RELATED CASES

2019-800-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

► Multiplex Epigenetic Editing using a Split-dCas9 System

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