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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

CONTACT

Prabakaran Soundararajan
psoundararajan@ucdavis.edu
tel: .



INVENTORS

- ▶ Deng, Peter
- ▶ Fink, Kyle
- ▶ Halmai, Julian

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

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University of California, Davis

Technology Transfer Office

1850 Research Park Drive, Suite 100, ,
Davis, CA 95618

Tel: 530.754.8649

techtransfer@ucdavis.edu

<https://research.ucdavis.edu/technology-transfer/>

Fax: 530.754.7620

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