

# Epigenetic Prevention and Treatment of CDKL5 Deficiency Disorder

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## 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	<a href="#">20220389393</a>	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](#)

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