

SELF-INACTIVATING TARGETED DNA NUCLEASES FOR GENE THERAPY

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PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,530,421	12/20/2022	2016-029

BRIEF DESCRIPTION

The clinical application of targeted nucleases - such as zinc-finger nucleases, TALENs, and CRISPR/Cas9 – are exciting genome editing platforms. Delivery of nucleases to cells and tissues using as viral methods, however, can leave the nucleases stably present in the target cells, even after editing has been accomplished. One major safety concern is off-target effects (i.e. cutting a non-intended site), which pose a safety risk. Another safety concern for gene therapies is the long-term expression of a foreign protein potentially provoking inflammatory reactions, another safety risk.

To avoid these potential detrimental outcomes, researchers at UC Berkeley have modified the delivered nuclease DNA which will cleave the host genome target DNA site and also excise its own DNA from the stable delivered construct. The researchers have shown that there is no trace of any active delivered DNA remaining, thus mitigating the harmful side effects from nuclease based gene therapy.

SUGGESTED USES

- » Clinical/research based gene therapy supplement

ADVANTAGES

- » Modification can be made to any deliverable nuclease system, including zinc finger nucleases, TALENs, and the CRISPR/Cas9 system
- » Increase safety of nuclease systems
- » Eliminates off-target effects
- » Removes long lasting exogenous material
- » Prevents immune recognition of the foreign proteins

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [Membrane-Associated Accessory Protein Variants Confer Increased AAV Production](#)
- ▶ [Human Central Nervous System \(CNS\) Targeting AAV Variants](#)
- ▶ [Improving Packaging and Diversity of AAV Libraries with Machine Learning](#)

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OTHER INFORMATION

KEYWORDS

gene editing, DNA nuclease

CATEGORIZED AS

- » **Biotechnology**
- » Genomics
- » **Medical**
- » Gene Therapy
- » Research Tools
- » Therapeutics

RELATED CASES

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