

# COMPOSITIONS AND METHODS FOR REDUCING RNA LEVELS

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## PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	WO 2023/250324	12/28/2023	2022-143

Additional Patent Pending

## BRIEF DESCRIPTION

Human diseases that follow a dominant negative inheritance pattern present a great challenge for treatment using gene therapy methods. In such cases, a copy of an allele is inherited from each parent: one is a pathogenic allele causing a disease phenotype (e.g., by exerting a toxic, gain-of-function effect) and the other is a wild-type (non-pathogenic) allele. Allele-specific targeting is especially important when the wild-type allele is crucial to normal function, e.g., the wild-type allele encodes a protein whose function is critical. There is therefore a need for compositions and methods of allele-specific gene editing.

UC Berkeley researchers have created methods and systems for reducing the level of an RNA transcript from a target nucleic acid in an allele-specific manner. Such systems and methods can be used to treat a disease that results from or is caused by a toxic gain-of-function protein.

## SUGGESTED USES

- » Reducing level of RNA transcript from a target nucleic acid
- » Treatment of diseases resulting from or caused by a toxic gain-of-function protein

## ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [Methods and Compositions for the Treatment of Huntington's Disease](#)

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## INVENTORS

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## OTHER INFORMATION

### CATEGORIZED AS

- » [Medical](#)
- » [Gene Therapy](#)
- » [Research Tools](#)
- » [Therapeutics](#)
- » [Research Tools](#)
- » [Nucleic Acids/DNA/RNA](#)

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