COMPOSITIONS AND METHODS FOR REDUCING RNA LEVELS

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PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20260085311	03/26/2026	2022-143
European Patent Office	Published Application	4544045	04/30/2025	2022-143

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

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OTHER INFORMATION

CATEGORIZED AS

- **Medical**
  - Gene Therapy
  - Research Tools
  - Therapeutics
- **Research Tools**
  - Nucleic Acids/DNA/RNA

RELATED CASES

2022-143-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Methods and Compositions for the Treatment of Huntington's Disease

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