RocASO: A Breakthrough Platform to Silence Undruggable RNA Targets and Transform Therapeutics

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TECHNOLOGY DESCRIPTION

UCSF inventors have developed a platform that represents a groundbreaking therapeutic approach to targeting undruggable RNA sequences by conjugating antisense oligonucleotides (ASOs) with Rocaglamide (RocA), a natural molecular glue. This innovative technology enhances ASO binding affinity to specific mRNA sequences and a protein that effectively blocks ribosomal attachment, preventing protein translation. RocASOs address the critical challenge of insufficient ASO potency in reducing protein levels, enabling the precise silencing of disease-driving genes in cancers, as well as potentially in neurodegenerative diseases, infectious diseases, and genetic disorders. By leveraging RocA's ability to specifically inhibit protein translation onto specific RNA sequences, the platform provides unmatched versatility for therapeutic development, including antiviral therapies, cancer treatments, and research tools for RNA-targeted drug discovery. This transformative technology offers a powerful solution to address unmet needs in precision medicine and revolutionize RNA therapeutics.

STAGE OF DEVELOPMENT

Validated through in vitro cell-line assays, RocASOs demonstrate increased efficacy, potency, and specificity relative to traditional antisense oligonucleotides (ASOs).

RELATED MATERIALS

- Programmable translational inhibition by a molecular glue-oligonucleotide conjugate. PMCID: PMC12338673; DOI: 10.1101/2025.07.15.664547 - 07/16/2025

PATENT STATUS

Patent Pending

CONTACT

Catherine Smith
Catherine.Smith2@ucsf.edu
tel: 510-646-0631.

OTHER INFORMATION

KEYWORDS

RNA-targeted drug discovery platform, Antisense oligonucleotide (ASO) conjugation technology, Therapeutic solutions for undruggable RNA sequences, Precision medicine for gene silencing in cancer and diseases, Rocaglamide (RocA)-enhanced RNA therapeutics

CATEGORIZED AS

- **Biotechnology**
  - Genomics
- **Medical**
  - Disease: Cancer
  - Disease: Infectious Diseases

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ADDRESS

UCSF

Innovation Ventures

600 16th St, Genentech Hall, S-272,

San Francisco,CA 94158

CONTACT

Tel:

innovation@ucsf.edu

https://innovation.ucsf.edu

Fax:

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