(SD2019-232) Technologies that can be Used to Selectively Bind Messenger RNA and Enhance Protein Translation

Tech ID: 30486 / UC Case 2019-232-2

BACKGROUND

Control of gene expression is a general approach to treat diseases where there is too much or too little of a gene product. However, while there are many methods which are available to downregulate the expression of messenger RNA transcripts, very few strategies can upregulate the endogenous gene product.

The vast majority of gene regulatory drugs which are commercially available or being developed are designed to knockdown gene expression (i.e. siRNAs, miRNAs, anti-sense, etc.). There exist some methods to enhance gene expression, such as the delivery of messenger RNAs; although, therapeutic delivery of such large and charged RNA molecules is technically challenging, inefficient, and may not be practical. There are also classical gene therapy approaches where a gene product is delivered as viral-encoded products (AAV or lentivirus-packaged).

However, these methods suffer from not being able to accurately reproduce the correct alternatively spliced isoforms in the right ratios in cells.

TECHNOLOGY DESCRIPTION

Researchers from UC San Diego have invented a two different approaches to leverage regulatory biology to enable the development of first-in-class gene targeting drugs that will selectively enhance protein translation. This technology encompasses **two technologies** that can be used to selectively bind messenger RNA and enhance protein translation. This technology can be targeted to any protein coding sequence encoded by the genome.

APPLICATIONS

- Reagent for Scientific Research. Reagents for controlled translation of a messenger RNA.
- Drug/Therapeutic to treat disease. Therapeutic delivery for the treatment of diseases rooted in haploinsufficiency

PATENT STATUS

Patent Pending

INTELLECTUAL PROPERTY INFO

UC San Diego is seeking companies interested in licensing related patent-pending technologies to develop commercial products.

Published PCT patent applications:

PROTEIN TRANSLATIONAL CONTROL using CAS polypeptide (2019-232-2)

In one aspect, provided herein are complexes comprising: a Cas polypeptide; and a capped-sgRNA comprising (i) an m7G cap or an analog thereof; (ii) a spacer capable of specifically hybridizing with a target sequence in an RNA molecule; and (iii) a direct

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OTHER INFORMATION

KEYWORDS

Protein translation, gene expression, mRNA

CATEGORIZED AS

- **Biotechnology**
  - Genomics
- **Medical**
  - Disease: Central Nervous System
  - Gene Therapy
  - Research Tools
  - Therapeutics
- **Research Tools**
  - Reagents

RELATED CASES

2019-232-2

repeat capable of binding to the Cas polypeptide. In some embodiments of any of the complexes described herein, the RNA molecule is a messenger RNA (mRNA). In some embodiments of any of the complexes described herein, the mRNA has an endogenous m7G cap. In some embodiments of any of the complexes described herein, the target sequence is downstream of the endogenous m7G cap of the mRNA.

https://patents.google.com/patent/WO2020214830A1/en?oq=US2020%2f028546 (2019-232-**2**)

PROTEIN TRANSLATIONAL CONTROL using oligonucleotides (2019-232-3)

In one aspect, provided herein is a cap-conjugated oligonucleotide comprising an m7G cap or a variant or analog thereof conjugated to an oligonucleotide, wherein the oligonucleotide is capable of specifically hybridizing with a target sequence in an RNA molecule. In some embodiments, the RNA molecule is a messenger RNA (mRNA). In some embodiments, the mRNA has an endogenous m7G cap. In some embodiments, the target sequence is downstream of the endogenous m7G cap of the mRNA.

https://patents.google.com/patent/WO2020214806A1/en?oq=US2020%2f028501 (2019-232-**3**)

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