(SD2019-040) Directed modification of cellular RNA via nuclear delivery of CRISPR/Cas

Tech ID: 32292 / UC Case 2019-040-0

BACKGROUND

Present strategies aimed to target and manipulate RNA in living cells mainly rely on the use of antisense oligonucleotides (ASO) or engineered RNA binding proteins (RBP). Although ASO therapies have been shown great promise in eliminating pathogenic transcripts or modulating RBP binding, they are synthetic in construction and thus cannot be encoded within DNA. This complicates potential gene therapy strategies, which would rely on regular administration of ASOs throughout the lifetime of the patient. Furthermore, they are incapable of modulating the genetic sequence of RNA. Although engineered RBPs such as PUF proteins can be designed to recognize target transcripts and fused to RNA modifying effectors to allow for specific recognition and manipulation, these constructs require extensive protein engineering for each target and may prove to be laborious and costly.

TECHNOLOGY DESCRIPTION

UC San Diego researchers' novel modification technology is largely built upon the existing RCas9 system, which provides a simple and rapidly programmable system for modification of specific RNA molecules in live cells. Due to its overall design simplicity as well as its highly encodable nature, this platform promises high utility and versatility when compared to other similar RNA modifying methods.

This technology pertains to compositions, systems, methods, and kits utilizing CRISPR-Cas protein fusions comprising a guide nucleotide sequence-programmable RNA binding protein and a RNA base modification protein.

APPLICATIONS

Research tool. Characterize the effects of directed cellular RNA m6A modification on RNA processing and cellular dynamics.

Therapeutic for diseases.. This could include diseases associated with deficiencies in RNA m6A modification.

The compositions, systems, methods, and kits described are useful to modulate RNA methylation and/or cytidine deamination.

This technology provides a method for modulating embryonic stem cell maintenance and/or differentiation, nervous system development, circadian rhythm, heat shock response, meiotic progression, DNA ultraviolet (UV) damage response, or XIST mediated gene silencing.

Also provided is a method for treating a disease or condition associated with m6 A RNA methylation of a target RNA in a subject in need thereof.

ADVANTAGES

STATE OF DEVELOPMENT

Researchers have generated prototypes of the Cas directed modification system that 1) recognize and edit a reporter mRNA construct in living cells at a base specific level and 2) mediate silencing of expression from reporter transcripts in cell culture.

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

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OTHER INFORMATION

KEYWORDS

epitranscriptomics, m6A effector, m6A methylation, m6A reader, m6A writer, mRNA modification

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Gene Therapy

RELATED CASES

2019-040-0

INTELLECTUAL PROPERTY INFO

RELATED MATERIALS

PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	2020/047498 A1	03/05/2020	2019-040

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