(SD2022-099) Repeat expansion disease therapy with antisense RNA vectors

Tech ID: 32922 / UC Case 2021-Z08-1

CONTACT
Skip Cynar
scynar@ucsd.edu
tel: 858-822-2672.

USER DEFINED 2

Researchers from UC San Diego have invented a RBP/RNP-recruiting motif that is a criticial component for the utility of the technology, which is able to recruit any combination of endogenous mammalian RBP/RNPs, including U snRNP components , hnRNP components, and other nuclear expressed RBP/RNPs. Without the motif, the nuclear expressed antisense RNA will lack the stability to target repetitive RNA effectively.

BACKGROUND

Alternative splicing accounts for a considerable portion of transcriptomic diversity, as most protein-coding genes are spliced into multiple mRNA isoforms. However, errors in splicing patterns can give rise to mis-splicing with pathological consequences, such as the congenital diseases familial dysautonomia, Duchenne muscular dystrophy, and spinal muscular atrophy. Small nuclear RNA (snRNA) components of the U snRNP family have been proposed as a therapeutic modality for the treatment of mis-splicing. U1 snRNAs offer great promise, with prior studies demonstrating in vivo efficacy, suggesting additional preclinical development is merited. Improvements in enabling technologies, including screening methodologies, gene delivery vectors, and relevant considerations from gene editing approaches justify further advancement of U1 snRNA as a therapeutic and research tool.

TECHNOLOGY DESCRIPTION

Researchers from UC San Diego have invented a RBP/RNP-recruiting motif that is a criticial component for the utility of the technology, which is able to recruit any combination of endogenous mammalian RBP/RNPs, including U snRNP components , hnRNP components, and other nuclear expressed RBP/RNPs. Without the motif, the nuclear expressed antisense RNA will lack the stability to target repetitive RNA effectively.

The genetic vector may be delivered into mammalian cells, animal models, or human patients with transfection reagents or in a viral vector such as lentivirus or adeno-associated virus (AAV).

APPLICATIONS

U1 snRNA as a candidate therapeutic and research tool.

OTHER INFORMATION

KEYWORDS

ASO, Alternative splicing, Antisense, Exon, Exonic, FAS, Gene therapy, RNA, NA targeting, Sequence-specific, Small nuclear, pre-mRNA, snRNA, snRNP, U snRNA, U1 snRNA, Uridine-rich, Splicing enhancer, Spliceosome, Splice site

CATEGORIZED AS

- **Biotechnology**
  - Genomics
  - Proteomics
- **Medical**
  - Gene Therapy
  - Research Tools
  - Therapeutics

RELATED CASES

2021-Z08-1

1) Research tool. The invention could be used to characterize the effects of repetitive RNA knockdown on

RNA, RBP, and cellular dynamics.

2) Therapeutic for diseases. The invention could be used as a therapeutic to treat diseases caused by and/or associated with repetitive RNA.

INTELLECTUAL PROPERTY INFO

UC San Diego is securing patent rights in this technology and seeking opportunities with industry to

commercialize the same.

RELATED MATERIALS

- Hatch ST, Smargon AA, Yeo GW. Engineered U1 snRNAs to modulate alternatively spliced exons. Methods. 2022 Sep;205:140-148. doi: 10.1016/j.ymeth.2022.06.008. Epub 2022 Jun 25 - 06/24/2022

University of California, San Diego

Office of Innovation and Commercialization

9500 Gilman Drive, MC 0910, ,

La Jolla,CA 92093-0910

Tel: 858.534.5815

innovation@ucsd.edu

https://innovation.ucsd.edu

Fax: 858.534.7345