Small Molecules to Facilitate Therapeutic Exon Skipping

Tech ID: 20445 / UC Case 2009-381-0

SUMMARY

Investigators at UCLA have discovered a series of small molecules to facilitate therapeutic exon skipping. The use of these identified molecules may enhance the effectiveness of antisense oligonucleotide agents currently in clinical development.

BACKGROUND

A number of antisense oligonucleotide agents are currently in clinical trials for a wide range of diseases. Antisense technology is broadly used by the pharmaceutical industry as a tool for functional genomics and for highly specific drugs in different therapeutic areas. Antisense oligonucleotides in clinical trials are frequently found to be too inefficient to cause a sufficient amount of exon skipping to be therapeutically effective. To date, no molecule that can increase the efficiency of antisense mediated skipping has been identified.

INNOVATION

Researchers at UCLA have discovered a series of compounds that facilitate therapeutic exon skipping. The compounds were derived from FDA approved libraries or known biologically active molecule libraries. The molecules were identified via a small molecule library screen using a cell reporter assay. Some compounds have been demonstrated to increase the amount of mRNA that is skipped in the presence of antisense therapeutics.

APPLICATIONS

- Enhancement of the therapeutic effect of antisense oligonucleotides when used as a combination treatment
- Increase in the amount of mRNA that is skipped in the presence of antisense therapeutics

ADVANTAGES

- Enhancement of the therapeutic effect of antisense treatments that are currently too inefficient to be effective
- The compounds were derived from FDA-approved libraries of known biologically active molecule libraries

STATE OF DEVELOPMENT

The compounds have been identified from a small molecule library screen using a cell reporter assay. Some compounds have undergone further testing in cell culture and are able to increase the amount of mRNA that is skipped in the presence of antisense entities.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	10,188,633	01/29/2019	2009-381

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OTHER INFORMATION

KEYWORDS

combination, antisense, oligonucleotides, exon, skipping, treatment, adjuvant

CATEGORIZED AS

- **Medical**
  - Therapeutics

RELATED CASES

2009-381-0

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