

## 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

### ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ [Small Molecules That Facilitate Exon Skipping](#)

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### INVENTORS

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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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