

# Enhanced gene activation through modification of small RNA duplexes

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## BRIEF DESCRIPTION

Small duplex RNAs have been shown to activate the expression of therapeutically relevant genes in a sequence-specific manner. UCSF researchers have identified chemical modifications and sequence features that enhance the activity and specificity of such duplex RNAs on targeted gene activation.

## FULL DESCRIPTION

Small duplex RNAs can trigger endogenous gene expression by targeting promoter-derived sequences and/or antisense transcripts. Such duplex RNAs are composed of 2 short (~21nt) RNA strands that bind each other *via* complementary base paring. One strand possesses the activity and sequence requirement to induce gene expression and is referred to as the “guide strand”. The alternate strand is referred to as the “passenger strand” and does not possess the required sequence for gene activation. By chemically modifying duplex RNAs, UCSF researchers have identified certain features that enhance function and specificity without interfering with activity. Such features include: (i) inhibiting the off-target effects of the passenger strand; (ii) enhancing target gene induction facilitated by the guide strand; (iii) defining benign modifications to the guide and/or passenger strand(s). Utilizing such modifications would avert some of the potential off-target effects of gene activation by duplex RNAs, as well as provide additional beneficial properties to improve its development as a laboratory tool.

## ADVANTAGES

- Improve specificity of activating RNA duplexes
- Reduce off-target effects
- Enhance activity
- Improve medicinal properties
- Predictably manipulate activating RNA duplex function

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## OTHER INFORMATION

### KEYWORDS

Small RNA

### CATEGORIZED AS

- ▶ [Medical](#)
- ▶ [Gene Therapy](#)
- ▶ [Therapeutics](#)

### RELATED CASES

2010-119-0

SUGGESTED USES

- Enhance the therapeutic potential of RNA-based drugs designed to activate gene expression in the treatment of human disease (i.e. cancer).
- Improve basic research experiments evaluating gene overexpression.
- Facilitate the synthesis of functional duplexes conjugated to small molecules (i.e. cholesterol, fluorogenic labels, etc.) to improve delivery or monitor cellular uptake.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	9,045,751	06/02/2015	2010-119

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