



**Non-Confidential  
Technology Disclosure**

- Title:** 5' phosphorylated, single-stranded siRNAs that trigger RNA interference
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- Description:** siRNA duplexes can trigger RNA interference (RNAi). Data suggests that the siRNA duplex is likely unwound during the RNAi pathway and that a 5' phosphate on the siRNA is critical to RNAi integrity. This invention demonstrates that single-stranded siRNAs can be used in RNAi *in vitro* or *in vivo* provided that they are 5' phosphorylated or phosphorylatable, which facilitates entry into the RNAi pathway. Further, modifications can be made in the 2' or 3' positions on the siRNA terminus or in the 5' end, provided that the siRNA molecule remains 5' phosphorylated.
- Application:** Single-stranded siRNAs also have potential applications in:
- Therapeutics for disorders that exhibit unwanted or aberrant gene expression or activity
  - Generation of knockout or knockdown phenotype in cells or non-human organisms
  - Functional genomics and/or proteomics
  - Reagent purposes in RNAi experiments
- Advantages:** This invention may allow for:
- Less expensive siRNA molecules, as single-stranded siRNAs are smaller and therefore cheaper to produce than duplexes
  - Designing anti-sense RNA oligonucleotides (single-stranded siRNAs) than can enhance siRNA stability, as chemical modifications at the 3' end of the single-stranded siRNAs does not affect its function.
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