Intellectual Property (Non-confidential)



# Method of Producing siRNA in Human Cells



# DESCRIPTION

RNA interference is the process of sequence-specific, posttranslational gene silencing initiated by double-stranded RNA that is homologous to the silenced gene. The use of small interfering RNA molecules, or siRNA, for gene silencing has profound utility in medicine; however, the lack of a reliable method for exogenous delivery of siRNA molecules to target cells has hampered the use of siRNA as a therapeutic agent. The present technology overcomes this limitation by providing methods for producing siRNA molecules in human cells. This method of producing siRNA molecules inside the target cell circumvents the difficult issue of siRNA delivery *in vivo* thereby increasing the therapeutic potential of siRNA to treat a variety of diseases.

# KEY ASPECTS

• Novel method of producing siRNA inside human cells circumvents significant problems associated with exogenous delivery of siRNA

#### **PUBLICATIONS**

- Lee NS, & Rossi JJ et al., Functional and intracellular localization properties of U6 promoter-expressed siRNAs, shRNAs, and chimeric VA1 shRNAs in mammalian cells. *RNA*. 2008 Sep;14(9):1823-33. PMID: 18697923.
- Li MJ, & Rossi JJ et al., Inhibition of HIV-1 infection by lentiviral vectors expressing Pol III-promoted anti-HIV RNAs. *Mol Ther.* 2003 Aug;8(2):196-206. PMID: 12907142.

## INTELLECTUAL PROPERTY

Title	Patent Number	Country	Date Issued
Methods for producing interfering RNA molecules in mammalian cells and therapeutic uses for such molecules	7,820,632	US	10/26/10
	2003/209,128 B2	AU	5/15/08
	1,483,281	EPO	9/3/08

\* Elected EPO countries include Germany, Spain, France, UK, Ireland

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