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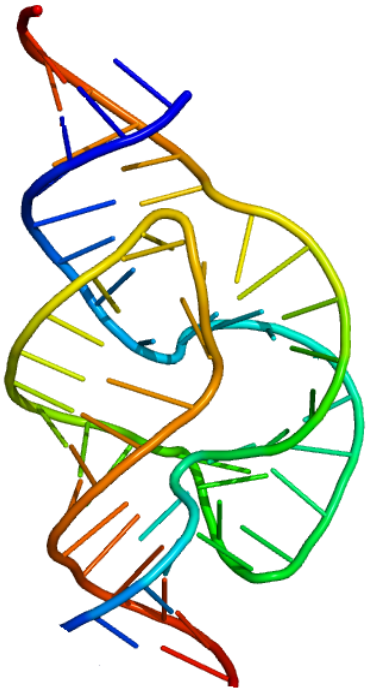
City of Hope
NATIONAL MEDICAL CENTER AND
BECKMAN RESEARCH INSTITUTE

Nucleolar Localization of Ribozymes for the Treatment of HIV



DESCRIPTION

A ribozyme is an RNA molecule with a well defined tertiary structure that enables it to catalyze a chemical reaction. Ribozyme means ribonucleic acid enzyme. It may also be called an RNA enzyme or catalytic RNA. It contains an active site that consists entirely of ribosomal RNA. Many natural ribozymes catalyze either the cleavage of one of their own phosphodiester bonds (self-cleaving ribozymes), or the cleavage of bonds in other RNAs. Examples of ribozymes include the hammerhead ribozyme, the VS ribozyme and the hairpin ribozyme.

This technology is the attachment of an anti-HIV agent to a snoRNA to deliver this agent to the nucleolus of an HIV infected cell where it will be in close contact with the virus and therefore deactivate the virus. The U16 snoRNA used in this technology is a vector to deliver an anti-HIV-1 hammerhead ribozyme directed to a conserved sequence of the HIV virus. The U16 snoRNA is also used as a vector to deliver a TAR element, which binds to and sequesters Tat in the nucleolus.

KEY ASPECTS

- Technique for targeting Ribozymes to nucleolus
- Therapeutic applications in HIV

INTELLECTUAL PROPERTY

Title	US Patent Number	Issued
Nucleolar Targeting of Therapeutics Against HIV	6,995,258	2/7/2006
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Nucleolar Targeting of Therapeutics Against HIV	12/835,333	7/13/2010

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