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Method for synthesizing therapeutic oligonucleotide conjugates with enhanced cellular uptake

The invention concerns a convenient synthesis method for oligonucleotide conjugates that are efficiently taken up into cells and can be used for antisense or siRNA-based therapies. The invention was made by researchers at the University of Vienna, Department for Pharmaceutical Chemistry.

BACKGROUND

Disease therapy through delivery of DNA or RNA oligonucleotides to cells is a highly promising technology, but its clinical application has been limited by various factors, including unfavourable pharmacokinetics and inefficient cellular uptake.

TECHNOLOGY

A new and convenient synthesis method has been developed for coupling a ligand (e.g. PEG, peptides) through a cleavable disulfide linkage to an oligonucleotide at the 2´ position of a ribose ring. The resulting oligonucleotide conjugates are taken up efficiently into cells without the need for a transfection agent, and the 2´ OH group modification has a positive impact on stability, allowing natural (phosphodiester) oligonucleotide sequences to be used. Preliminary *in vitro* experiments have demonstrated effective target gene down-regulation.

ADVANTAGES

- Technology platform for facile synthesis of oligonucleotide conjugates with labile linkages to selected ligands.
- No evidence of non-specific cellular toxicity/apoptosis.
- Cellular Uptake is efficient and the oligonucleotides have good bioavailability and efficacy *in vitro.*
- No need to use modified (e.g. phosphorothioate) oligonucleotides: conjugates based on natural oligonucleotides are sufficiently stable.

APPLICATIONS:
Treatment of varied medical conditions using antisense or siRNA therapy.

DEVELOPMENT STATUS:
Proof of concept *in vitro*

KEYWORDS:
- Gene therapy
- Oligonucleotides
- Antisense
- RNAi
- siRNA
- Aptamers

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