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Nanoconstructs for delivery of RNA splice-switching oligonucleotide therapeutics (NanoSplice)

Oligonucleotides are increasingly being researched for use in gene therapy, mainly to inactivate gene expression using the antisense or ribozyme approaches. However, this strategy suffers from limited efficacy due to inefficient uptake of the oligonucleotides by target cells. The large oligonucleotide doses that are needed induce side effects and render the treatment highly costly. Our project aims at developing nano-tools for increasing the efficacy of therapeutic nucleic acids by adding small address labels that direct them to cell nuclei, and also by changing their chemical nature, so that they more readily are taken up into cells.