

Nano Science, Technology and Industry Scoreboard

Overcoming Challenges in the Delivery of Nucleic Acid Therapeutics

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Nucleic acid therapies involving DNA or RNA have significant potential to treat genetic disorders, infectious diseases, and cancer; however, research suggests that less than 1% of injected nucleic acid doses reach target cells in an active form. An article in WIRES Nanomedicine and Nanobiotechnology highlights two of the major barriers to this therapeutic strategy, and how they might be overcome.

Delivery to the target cell and transport to the sub-cellular compartment where the nucleic acids are therapeutically active represent the most significant challenges to address. The authors explore how nanoparticle delivery systems can be modified with targeting molecules to increase accumulation in specific cells, and how the composition of the nanoparticle can be engineered to manipulate or disrupt cellular membranes and facilitate delivery to the optimal sub-cellular compartments.

"Controlling the sub-cellular delivery of DNA and RNA is the next major frontier for biotherapeutics. If we can overcome these barriers, DNA and RNA technology has the potential to revolutionize the treatment of a range of diseases," said corresponding author Angus P.R. Johnston, PhD, of Monash University, in <u>Australia</u>.

Read the <u>original article</u> on Wiley.