

# Towards safe nanoparticle technologies for nucleic acid therapeutics

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## ABSTRACT

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Nucleic acid therapeutics (or gene therapy) has to date failed to deliver on promise but rapid improvements in the understanding and use of delivery technologies should reverse this situation. In this review of work performed in and in collaboration with the Imperial College Genetic Therapies Centre, progress towards safe nanoparticles for efficient delivery of functional nucleic acids *in vivo* is described. The intention is to demonstrate the fruits of a journey from the results of initial studies in animal models of disease that suggested that so much should be possible so quickly, to the realization that new technologies are rarely successful so quickly, through to developments in the present day that appear to be approaching the preclinical/clinical threshold with realism but measured confidence. New chemistry is central to the design and formulation of safe nanotechnologies. Chemistry should have a central role to play in ensuring that nucleic acid therapeutics truly live up to their potential for therapy and cure, none more so than in the derivation of newer and better therapies for cancers.

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**Key words:** *nanoparticles, nucleic acid delivery, non-viral vectors, gene therapy.*

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