PRINCIPE FELIPE
CENTRO DE INVESTIGACION

Doctoral Thesis

Well-defined polypeptide-based systems as non-viral vectors for cytosolic delivery

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Abstract: A convenient cytosolic drug delivery constitutes a very powerful tool for the treatment and/or prevention of several relevant human diseases. Along with recent advances in therapeutic technologies based on biomacromolecules (*e.g.* oligonucleotides or proteins), we also require the development of technologies which improve the transport of therapeutic molecules to the cell of choice. In this context, Polymer Therapeutics (PT) have emerged as an exciting alternative to overcome such limitations. Specifically, well defined polypeptide-based therapeutics could be considered excellent candidates for drug delivery due to their suitable biodegradability, versatility, multivalence and high drug loading capacity. On the other hand, a comprehensive understanding of therapeutic molecules is also required for the rational selection and design of an appropriate intracellular delivery carrier. On this basis, the main aim of this thesis was focused on two main topics: (i) the design, development, and validation of nanosized polypeptide-based carriers capable of facilitating the cytosolic transport of bioactive agents which are not able to cross biological membranes by themselves or exhibit low lysosomal stability, such as plasmid DNA (pDNA), small interfering RNA (siRNA), or proteins, and (ii) the exhaustive physicochemical characterisation of polymeric drug delivery systems to determine their solution conformation and its correlation with their therapeutic output.

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