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Drug Delivery of siRNA Therapeutics

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The new frontier of pharmaceutical sciences is gene therapy, which is the use of molecules able to interact directly with the expression of the genetic material of the patient as well as of the disease-causing guest (bacteria, virus, parasites, and tumor cells). Among the molecules of interest for gene therapy, a relevant role is played by small interfering RNA (siRNA) molecules able to interfere with the expression of genes of interest for some diseases. However, siRNA molecules, even if they are powerful as drugs, are difficult to deliver since they are sensitive to enzymes present in plasma and they are large and negatively charged, so are difficult to administer into the cell nuclei, since the cell walls are scarcely permeable to large molecules and are also negatively charged. Therefore, the focus of research on siRNA-based therapies is their delivery, which can be performed by chemical modification, association with aptamers or polycations, or embedding them into properly designed liposomes. This book is centered on the more recent development in siRNA delivery techniques toward the clinical applications of this potent class of drugs.

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