

Advancements and challenges in nonviral gene therapeutics: A physical chemist's perspective

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The use of lipid-mRNA vaccines for COVID-19 has brought unprecedented success and highlighted the potential of nonviral gene therapeutics. However, this achievement is the culmination of over three decades of interdisciplinary efforts in biology, chemistry, and physics [1]. Despite this progress, the field still faces significant challenges in delivering genes to cells for the treatment of a wide range of diseases.

In this talk, we will provide an overview of the main aspects of nonviral gene therapeutics, with a focus on the physical chemistry of lipid-based DNA formulations. We will highlight our own contributions to the field, specifically the use of Small-Angle X-ray Scattering (SAXS) [2] and Fluorescence Cross-Correlation Spectroscopy (FCCS) [3,4] to understand the structure and organization of these formulations. By leveraging these techniques, we can gain insight into the fundamental behavior of lipid-DNA nanoparticles and improve their design for more effective gene delivery.

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Nanomedicine for the treatment of pediatric cancer

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This talk is about the use of nanomedicines for pediatric cancer in the light of the dismal current situation in childhood cancer management. Compared to the situation with adults, agencies, governments and institutions have pointed to an alarming gap concerning the arrival of novel approaches in children. Indeed, chemotherapy protocols for childhood cancer are still problematic due to the high toxicity associated with chemotherapeutic agents and incorrect dosing extrapolated from adults. Childhood cancers, like adult cancers, have to receive special attention with a view to developing novel therapies that are fit for purpose [1].

Osteosarcoma is the most frequent primary bone tumor in the pediatric population and one of the most frequent causes of pediatric cancer death. Due to its aggressive local growth pattern and its high propensity to metastasize, mainly to the lungs, it represents one of the leading causes of pediatric cancer death [2].

Osteosarcoma treatment is based on a neo-adjuvant multiagent chemotherapy followed by a surgical resection and adjuvant chemotherapy. Methotrexate, doxorubicin, ifosfamide and cisplatin combinations have shown the highest activity treating the non-metastatic disease [3].

During the past decades the advances in osteosarcoma treatment have been scarce without a significant improvement in survival. For instance, although doxorubicin is used as a first-line antineoplastic agent in the treatment of osteosarcoma, its narrow therapeutic window, severe adverse effects and the development of multidrug resistances have led researchers to investigate alternative forms of administering doxorubicine for cancer therapy [4].

The focus of this talk will be to discuss the potential of nanosystems loaded with edelfosine [5], a new antitumor agent, that has shown efficacy in several cancer cell lines [6,7], in the treatment of osteosarcoma [8,9].

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