Contribution ID: **90** Type: **Invited**

Messenger RNA-based nanomedicines: where are we from now ?

Tuesday, 14 March 2023 14:10 (40 minutes)

The field of nanomedicine has reached a milestone since the approval of Patisiran the first ever approved siRNA drug designed to treat transthyretin (TTR) amyloidosis and that of Corminaty and Spikevax messenger RNA (mRNA)-based vaccines against COVID-19, both delivered by a lipid nanocarrier. Nucleic acids are now considered as real hope but not only hype to cure unmet medical diseases as well as chronic diseases. As for any types of drugs, nucleic acids-based nanoparticles can be produced using different types of formulations made with polymer, inorganic materials protein/peptide derivatives and lipids. This talk will be focused on mRNA-based formulations mainly those made with lipid- based nanoparticles. Those formulations are quite challenging due to the peculiar nature of mRNA. Others and we have proposed strategies to cross multiple biological barriers including the plasma and intracellular membranes. I will present what is known so far in terms of efficacy of those strategies. Another challenge is to get a targeted delivery which could be reached either by manipulating the lipids composition or using targeting ligands for specific receptors. Issues that we have to face when conducting those two strategies will be discussed. It is becoming clear that we need a multidisciplinary approach to achieve a rational design of nanomedicine. Today, we can apply several available computational methodologies as accelerated modular-orthogonal methodology for formulations design, cell modelling, pharmacokinetic modelling, and computational toxicology associated with relevant high-throughput synthesis and analysis methods.

Session

Nanomedecine

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Session Classification: Nanomedecine