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Design of lipid nanoparticles to enable gene therapies

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Delivery of nucleic acid-based drugs into target cells in vivo has been a major challenge for enabling gene therapies. This barrier is now being overcome due in part to advances made in lipid nanoparticle (LNP) delivery systems. LNP systems enable the mRNA COVID-19 vaccines and there are a host of LNP RNA vaccines and therapeutics in clinical development. Advantages of LNP RNA systems over other delivery vectors include safety, ability to re-dose, essentially unlimited genetic cargo, ease of design, straightforward manufacturing processes, lower cost, and potential for highly personalized targeted therapeutics that can be developed in a matter of weeks. In this talk I will discuss the evolution of lipid nanoparticles and lead examples of therapeutic applications of LNP RNA systems to treat cancer, cardiovascular disease and rare diseases.

Keyword-1

Keyword-2

Keyword-3

Author: CULLIS, Pieter (University of British Columbia)

Presenter: CULLIS, Pieter (University of British Columbia)

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