OLIGO (SERINE ESTER) CHARGE ALTERING RELEASABLE TRANSPORTERS FOR DELIVERY OF mRNA

Abstract: In vitro transcribed-messenger RNA (IVT-mRNA) is an emerging class of nucleic acid therapeutics that serve as a promising treatment for genetic diseases. However, the delivery of IVT-mRNA in vivo has hampered its applications in clinical development. Work is still ongoing to find more dynamic delivery vector with high transfection activity, biocompatibility with minimal toxicity effect, and high selectivity and specificity. Recently, Benner, et. al developed oligo (serine ester)-based charge altering releasable transporters (ser-CARTs) for mRNA delivery. This new class of biodegradable gene delivery vectors are cationic hence forms stable complexes with polyanionic mRNA but degrades at physiological pH thereby efficiently releasing their payload. Benner et. al demonstrated that ser-CARTs can effectively deliver IVT-mRNA and that the protein encoded by the ser-CART delivered IVT-mRNA is sufficiently expressed both in vitro and in vivo. The development of ser-CARTs establishes a class of charge altering vehicles for gene delivery and have the potential to increase the utility of IVT-mRNA as a therapeutic strategy.

Reference