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In vivo transfection efficiencies and pharmacokinetics of mRNA delivered in naked and nanoparticle format

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As an emerging class of genetic medicine, pharmacokinetics of messenger RNA (mRNA) has been poorly characterized. We evaluated transfection efficiencies of mRNA in naked and nanoparticle format *in vitro* and *in vivo*. mRNA nanoparticles transfected both primary human and mouse DCs highly efficiently *in vitro* while naked mRNA did not. *In vitro* expression peaks rapidly 5-7th hours and decays in a biphasic manner. Naked mRNA transfects much more efficiently than mRNA nanoparticles *in vivo* at subcutaneous sites and is pH dependent. On the other hand, only mRNA delivered in nanoparticle form transfected when administered through intranasal and intravenous routes. Gene expression is the most transient when delivered intravenously with an apparent half-life of 1.4hours and lasts for less than 1 day. In contrast, gene expression is the most sustained when delivered in naked format subcutaneously at the base of tail with an apparent half-life of 18hours and lasts for at least 6 days. Interestingly, consistent exponential decreases in gene expression are observed when mRNA is delivered in both naked and nanoparticle formats at all sites of administration. Our data suggest a niche for mRNA therapeutics delivered in nanoparticle format administered through intranasal and intravenous routes, and predictable *in vivo* mRNA pharmacokinetics.

Biography

Kyle holds a B.Eng (Chemical Engineering) from National University of Singapore and a M.S (Chemical Engineering) from University of Illinois-Urbana-Champaign. Kyle won an academic scholarship from the National University of Singapore to pursue a PhD in Biomedical Engineering at Duke University under the supervision of Dr Kam Leong. He is currently investigating mRNA delivery strategies to transfect, activate and enhance mRNA tumor vaccination.

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