This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Lipopeptide nanoparticles (LPNs) for potent <i>in vivo</i> delivery of siRNA selectively to hepatocytes	Rodent and nonhuman primate studies suggest LPNs could be used to deliver siRNAs selectively to hepatocytes. Synthetic lipoamino acid derivatives formulated in nanoparticles with siRNA were screened in mice for target gene knockdown in the liver, yielding a lead LPN containing a dilysine-derived diketopiperazine core and four amino acid, alcohol-based lipid tails. In mice, siRNAs targeting three widely expressed genes delivered with the lead LPN led to selective gene silencing in hepatocytes. In nonhuman primates, i.v. injection of siRNA targeting <i>transthyretin</i> (<i>TTR</i>) formulated in the lead LPN led to knockdown of <i>TTR</i> mRNA with an ED ₅₀ of ~0.002 mg/kg. Next steps could include further elucidating the mechanism of LPN uptake by hepatocytes.	Patent filed; available for licensing from Alnylam Pharmaceuticals Inc.	Dong, Y. <i>et al. Proc. Natl. Acad. Sci. USA</i> ; published online Feb. 10, 2014; doi:10.1073/pnas.1322937111 Contact: Daniel G. Anderson, Massachusetts Institute of Technology, Cambridge, Mass. e-mail: dgander@mit.edu
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