

This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
<i>In vivo</i> delivery of microRNA antagonists	<p>A study in mice and primates suggests that unconjugated locked nucleic acid (LNA)-modified oligonucleotides could be superior to unmodified oligonucleotides for antagonizing miRNAs and treating disease. In hypercholesterolemic mice, twice-weekly administration of LNA-modified anti-miR-122 reduced total plasma cholesterol compared with levels in mice that received LNA-mismatch or saline controls. In African green monkeys, thrice-weekly administration of LNA-modified anti-miR-122 lowered total plasma cholesterol compared with levels in monkeys that received saline control. miR-122 is expressed in the liver and is implicated in cholesterol metabolism, lipid metabolism and HCV replication. Next steps include optimizing the dosing regimen and studying long-term safety in animals before testing the miRNA antagonists in humans.</p> <p>Santaris Pharma A/S plans to start a Phase I safety and pharmacokinetics trial of SPC3649, an LNA-anti-miR targeting miR-122 to treat HCV, in healthy volunteers this year.</p>	<p>miRNA silencing method patented by Santaris; GlaxoSmithKline plc has an option to license SPC3649 from Santaris under a 2007 agreement; method unavailable for licensing</p>	<p>Elmen, J. <i>et al. Nature</i>; published online March 27, 2008; doi:10.1038/nature06783 Contact: Sakari Kauppinen, Santaris Pharma, Horsholm, Denmark e-mail: sk@santaris.com</p>