This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Cardiomyopathy	Myosin heavy chain 7 cardiac muscle- β (MYH7)	<p>Mouse studies suggest RNAi that targets a disease-causing mutation in MYH7 could help prevent hypertrophic cardiomyopathy (HCM). The R403Q mutation in MYH7, which encodes the myosin heavy chain, causes progressive and early onset HCM. In mice with a heterozygous, homologous mutation, injection of an adenoviral vector encoding RNAi that specifically targets the mutant gene under a cardiac-specific promoter prevented both hypertrophy and fibrosis, whereas injection of a vector encoding control RNAi did not. Next steps include designing a modified adenoviral vector for delivery to the human heart.</p> <p><i>SciBX</i> 6(43); doi:10.1038/scibx.2013.1223 Published online Nov. 7, 2013</p>	Patent application filed; unavailable for licensing	<p>Jiang, J. <i>et al. Science</i>; published online Oct. 4, 2013; doi:10.1126/science.1236921 Contact: Christine E. Seidman, Harvard Medical School, Boston, Mass. e-mail: cseidman@genetics.med.harvard.edu</p>