This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Spinal and bulbar muscular atrophy (SBMA)	MicroRNA-196a (miR-196a)	<p>Studies in cell culture and in mice suggest viral delivery of miR-196a could help treat SBMA. In cell culture, an adeno-associated virus (AAV) vector bearing transgenic miR-196a, compared with an empty control vector, decreased expression of the polyglutamine-expanded mutant version of androgen receptor, which causes SBMA. In a mouse model of SBMA, peripheral injection of an miR-196a-expressing AAV vector improved gait and increased body weight and survival compared with injection of a nonspecific miRNA-expressing AAV vector. Next steps include optimizing a dosing and delivery route for the virus in a mouse model.</p> <p>SciBX 5(26); doi:10.1038/scibx.2012.685 Published online June 28, 2012</p>	Patent pending; available for licensing	<p>Miyakazi, Y. <i>et al. Nat. Med.</i>; published online June 3, 2012; doi:10.1038/nm.2791</p> <p>Contact: Gen Sobue, Nagoya University Graduate School of Medicine, Nagoya, Japan e-mail: sobueg@med.nagoya-u.ac.jp</p>