This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Ataxia	Frataxin (FXN; FRDA)	<p>Mouse studies suggest <i>FXN</i> gene therapy could help prevent or treat heart failure in patients with Friedreich's ataxia. In presymptomatic mouse models of Friedreich's ataxia, an i.v. injected adeno-associated virus serotype 10 (AAV10) vector encoding <i>FXN</i> prevented the onset of left ventricular hypertrophy, loss of cardiac function and death from heart failure that were observed in untreated mice. In symptomatic mouse models of Friedreich's ataxia, the AAV10-<i>FXN</i> gene therapy decreased multiple symptoms, including left ventricular hypertrophy and cardiac fibrosis, and increased cardiac function and survival compared with no treatment. Ongoing work includes testing the dose response of the gene therapy in mice.</p> <p>SciBX 7(16); doi:10.1038/scibx.2014.466 Published online April 24, 2014</p>	Patented by Cornell University and Institut National de la Santé et de la Recherche Médicale (INSERM); licensed to AAVLife	<p>Perdomini, M. <i>et al. Nat. Med.</i>; published online April 6, 2014; doi:10.1038/nm.3510</p> <p>Contact: Hélène Puccio, Institute of Genetics and Molecular and Cellular Biology, Illkirch, France e-mail: hpuccio@igbmc.fr</p>