

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Other				
Hearing loss	Usher syndrome 1C (USH1C)	<p>Mouse studies suggest using antisense oligonucleotides (ASOs) that correct splicing of mutant USH1C transcripts could help prevent Usher syndrome-associated hearing loss. In a neonatal mouse model of Usher syndrome, a single intraperitoneal injection of a splice-correcting ASO prevented development of auditory and vestibular defects, whereas injection of a control ASO did not. Next steps include optimizing the lead ASO and determining the best dosing regimen in collaboration with Isis Pharmaceuticals Inc.</p> <p>SciBX 6(7); doi:10.1038/scibx.2013.171 Published online Feb. 21, 2013</p>	Patents pending covering ASOs targeted to the mutant USH1C; licensing status undisclosed	<p>Lentz, J.J. <i>et al. Nat. Med.</i>; published online Feb. 4, 2013; doi:10.1038/nm.3106 Contact: Michelle L. Hastings, Rosalind Franklin University of Medicine and Science, Chicago, Ill. e-mail: michelle.hastings@rosalindfranklin.edu</p>