This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Musculoskeletal disease	Adenylate cyclase-stimulating G α protein (GNAS); glioma-associated oncogene homolog 1 zinc finger protein (GLI1)	<i>In vitro</i> and mouse studies suggest hedgehog pathway inhibitors could help treat heterotopic ossification caused by loss-of-function <i>GNAS</i> mutations. In mice, knockout of <i>Gnas</i> in mesenchymal progenitor cells upregulated hedgehog signaling compared with wild-type <i>Gnas</i> expression and induced heterotopic ossification. In the mouse model, knockout or pharmacological inhibition of <i>Gli1</i> , a hedgehog signaling effector, decreased heterotopic ossification compared with no <i>Gli1</i> knockout or inhibition. Next steps include testing hedgehog pathway antagonists in nongenetic preclinical models of heterotopic ossification and assessing their safety.	Patent application filed; available for licensing	Regard, J.B. <i>et al. Nat. Med.</i> ; published online Sept. 29, 2013; doi:10.1038/nm.3314 Contact: Yingzi Yang, National Institutes of Health, Bethesda, Md. e-mail: yingzi@mail.nih.gov
<i>SciBX</i> 6(42); doi:10.1038/scibx.2013.1195 Published online Oct. 31, 2013				