This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Musculoskeletal disease	FYVE, RhoGEF and PH domain containing 1 (FGD1); mitogen-activated protein kinase kinase kinase 11 (MAP3K11; MLK3)	<p>Studies in mice and in patient samples suggest modulating MLK3 could help treat skeletal defects in Aarskog-Scott syndrome, an X-linked genetic disorder caused by mutations in <i>FGD1</i>.</p> <p>In samples from five patients, expression of wild-type <i>FGD1</i> increased <i>MLK3</i> levels compared with expression of mutant <i>FGD1</i>. Compared with wild-type controls, mice lacking <i>MLk3</i> or carrying a mutant <i>Mtk3</i> allele resistant to activation by <i>Fgd1</i> had impaired bone mineralization and increased spontaneous tooth fracture. Next steps include developing agonists specific for MLK3 or downstream kinases and seeking additional targets in the signaling network that could modulate bone mass.</p> <p>SciBX 4(41); doi:10.1038/scibx.2011.1149 Published online Oct. 20, 2011</p>	Unpatented; available for licensing	<p>Zou, W. <i>et al. J. Clin. Invest.</i>; published online Oct. 3, 2011; doi:10.1172/JCI59041</p> <p>Contact: Laurie H. Glimcher, Harvard School of Public Health, Boston, Mass. e-mail: lglimche@hsph.harvard.edu</p>