



This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal	disease			
Musculoskeletal disease	Fibroblast growth factor receptor 3 (FGFR3; CD333)	In vitro and mouse studies suggest statins could help treat achondroplasia, thanatophoric dysplasia type 1 (TD1) and other skeletal dysplasias caused by FGFR3 mutations. Chondrocytes differentiated from induced pluripotent stem (iPS) cells derived from patients with achondroplasia or TD1 exhibited less proliferation and cartilage formation and greater apoptosis than chondrocytes differentiated from healthy control–derived iPS cells. In the patient-derived chondrocytes, statin therapy decreased mutant FGFR3 levels and cartilage degradation compared with vehicle. In a transgenic FGFR3-mutant mouse model of achondroplasia, the statin Crestor rosuvastatin increased degradation of mutant FGFR3 and bone growth compared with vehicle. Next steps include determining the best statin for additional testing.	Patent application filed; available for licensing	Yamashita, A. et al. Nature; published online Sept. 17, 2014; doi:10.1038/nature13775 Contact: Noriyuki Tsumaki, Kyoto University, Kyoto, Japan e-mail: ntsumaki@cira.kyoto-u.ac.jp
		SciBX 7(40); doi:10.1038/scibx.2014.1185 Published online Oct. 16, 2014		