This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Pulmonary disease				
Cystic fibrosis (CF)	Cystic fibrosis transmembrane conductance regulator (CFTR)	Cell culture studies suggest long-term studies with CFTR potentiator compounds could help assess their impact on mutant forms of the ion channel protein. In cultured human primary bronchial epithelial cells (HBEs) that express the CF-associated CFTR ΔF508, chronic application of the CFTR potentiator Kalydeco ivacaftor for 48 hours in combination with a corrector compound led to decreased CFTR ΔF508 stability, plasma membrane expression and channel activity compared with application of the corrector compound alone. In HBEs expressing wild-type CFTR, ivacaftor destabilized the channel. Next steps include assessing the effects of CFTR potentiator compounds on other mutant CFTRs and screening for potentiators that do not destabilize CFTR ΔF508. Vertex Pharmaceuticals Inc. markets Kalydeco ivacaftor to treat patients with CF carrying CFTR mutations that affect channel gating. *SciBX* 7(33); doi:10.*1038*/scibx.2014.992 Published online Aug. 28, 2014	Findings for both studies unpatented; licensing status not applicable	Veit, G. *et al. Sci. Transl. Med.*; published online July 23, 2014; doi:10.1126/scitranslmed.3008889 **Contact:** Gergely L. Lukacs, McGill University, Montreal, Quebec, Canada e-mail: gergely.lukacs@mcgill.ca Cholon, D.M. *et al. Sci. Transl. Med.*; published online July 23, 2014; doi:10.1126/scitranslmed.3008680 **Contact:** Martina Gentzsch, The University of North Carolina at Chapel Hill, Chapel Hill, N.C. e-mail: gentzsch@med.unc.edu