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)	<p><i>In vitro</i> and cell culture studies identified the molecular mechanism of action for CFTR corrector compounds and suggest new combination approaches that could help treat CF. In reconstituted phospholipid bilayers carrying recombinant $\Delta F508$ CFTR, the corrector compound VX-809 directly bound the protein and stabilized the interface between nucleotide binding domain 1 and membrane spanning domains 1 and 2. In cultured cells and samples from patients with CF that have the $\Delta F508$ CFTR mutation, VX-809 plus either of two compounds that help stabilize protein folding increased functional CFTR expression compared with any individual treatment. Next steps include identifying drug-like stabilizers of NBD1 folding, which could be combined with VX-809 or other CFTR correctors. Vertex Pharmaceuticals Inc.'s VX-809, a CFTR corrector, is in Phase III trials to treat $\Delta F508$ mutant CF in combination with the CFTR potentiator Kalydeco ivacaftor (VX-770). Vertex's VX-661, a CFTR corrector that is structurally related to VX-809, is in Phase II trials in combination with Kalydeco to treat $\Delta F508$ CF. Vertex markets Kalydeco to treat CF.</p> <p>SciBX 6(22); doi:10.1038/scibx.2013.553 Published online June 6, 2013</p>	Patent and licensing status unavailable	<p>Okiyoneda, T. <i>et al. Nat. Chem. Biol.</i>; published online May 12, 2013; doi:10.1038/nchembio.1253 Contact: Gergely L. Lukacs, McGill University, Montreal, Quebec, Canada e-mail: gergely.lukacs@mcgill.ca</p>