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THE DISTILLERY

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)	*In vitro* studies suggest stabilizing two distinct steps of mutant ΔF508 CFTR protein folding could help treat CF. *In vitro* studies showed the ΔF508 mutation in CFTR disrupts two protein-folding steps and leads to CFTR degradation. Computational and thermodynamic analyses of ΔF508 CFTR identified two sets of suppressor mutations that synergized to correct CFTR folding and restore protein function to wild-type levels. Next steps include screening for compounds that affect each CFTR folding step and identifying which folding step is affected by existing CFTR-targeted compounds. Vertex Pharmaceuticals Inc.'s VX-809, a CFTR corrector, is in Phase II trials to treat ΔF508 CF. Vertex's VX-661, also a CFTR corrector, is in preclinical development to treat ΔF508 CF. *SciBX* 5(6); doi:10.1038/scibx.2012.164 Published online Feb. 9, 2012	Findings for both studies unpatented; licensing status not applicable	Rabeh, W.M. *et al. Cell*; published online Jan. 20, 2012; doi:10.1016/j.cell.2011.11.024 **Contact:** Gergely L. Lukacs, McGill University, Montreal, Quebec, Canada e-mail: gergely.lukacs@mcgill.ca Mendoza, J.L. *et al. Cell*; published online Jan. 20, 2012; doi:10.1016/j.cell.2011.11.023 **Contact:** Philip J. Thomas, The University of Texas Southwestern Medical Center, Dallas, Texas e-mail: philip.thomas@utsouthwestern.edu