This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Various				
Cystic fibrosis (CF); muscular dystrophy	Ribosome	*In vitro* studies have questioned the mechanism of action for ataluren (PTC124), which is in clinical development to treat CF and Duchenne muscular dystrophy (DMD). The proposed mechanism of action for ataluren is to induce readthrough of premature termination codons (PTCs) by ribosomes, thus increasing protein expression in genetic disorders for which PTCs cause disease. In a series of *in vitro* assays to measure PTC readthrough, ataluren had no effect, whereas the aminoglycoside antibiotic geneticin had a dose-dependent response. Next steps could include studies designed to further explore the mechanism of action for drugs thought to induce PTCs *in vivo*. PTC Therapeutics Inc. has ataluren in Phase III testing to treat nonsense mutation DMD. Last year, the EMA accepted for review an MAA seeking conditional approval of ataluren to treat nonsense mutation DMD. The product also has completed a Phase III trial to treat nonsense mutation CF, with a confirmatory Phase III trial in the indication expected to start next year. *SciBX* 6(29); doi:10.1038/scibx.2013.767 Published online Aug. 1, 2013	Patent and licensing status not applicable	McElroy, S.P. *et al. PLoS Biol.*; published online June 25, 2013; doi:10.1371/journal.pbio.1001593 **Contact:** Stuart P. McElroy, University of Dundee, Dundee, U.K. e-mail: s.mcelroy@dundee.ac.uk