This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cancer				
Cancer	p53	<p><i>In vitro</i> and cell-based studies suggest combining an inhibitor of nonsense-mediated RNA decay and a promoter of premature stop-codon read-through could help treat cancer. Virtual library screening and <i>in vitro</i> testing identified several nonsense-mediated RNA decay inhibitors with nanomolar potency. In human cancer cell lines harboring premature stop-codon mutations in <i>p53</i>, one inhibitor increased mutant <i>p53</i> mRNA levels compared with vehicle. In the cancer cell lines, the inhibitor and a research compound that promotes premature stop-codon read-through synergistically increased functional <i>p53</i> levels and cell death compared with either agent alone. Next steps include optimizing the nonsense-mediated RNA decay inhibitors.</p> <p>PTC Therapeutics Inc.'s Translarna ataluren (formerly PTC124), a small molecule that facilitates complete translation of proteins containing nonsense mutations, is under EMA review to treat muscular dystrophy. The compound also is in Phase III testing to treat cystic fibrosis (CF) and Phase II trials to treat methylmalonic acidemia.</p> <p>SciBX 7(17); doi:10.1038/scibx.2014.485 Published online May 1, 2014</p>	Patented by New York University; unlicensed; available for partnering	<p>Martin, L. <i>et al. Cancer Res.</i>; published online March 24, 2014; doi:10.1158/0008-5472.CAN-13-2235 Contact: Lawrence B. Gardner, New York University School of Medicine, New York, N.Y. e-mail: lawrence.gardner@med.nyu.edu</p>