This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Pulmonary disease				
Cystic fibrosis (CF); muscular dystrophy; mucopolysaccharidosis I (MPS-I; Hurler's syndrome); Usher syndrome	Nonsense mutations causing premature translation termination	<i>In vitro</i> studies suggest that a new aminoglycoside derivative could help treat genetic diseases caused by nonsense mutations. In an <i>in vitro</i> assay using DNA fragments containing nonsense mutations found in Usher syndrome, CF, Duchenne muscular dystrophy and Hurler's syndrome, the derivative was more effective at reading through mutant stop codons than a predecessor compound, gentamicin, or paromomycin. In three kidney cell lines, in cochlear explants and in mice, the derivative showed lower toxicity than the other two compounds. Ongoing studies are testing the lead compound in a mouse model of CF. Next steps could also include studying the effects on animal models of the other diseases.	Findings patented; available for licensing from Technion-Israel Institute of Technology for the treatment of diseases in which nonsense mutation is an underlying cause	Nudelman, I. <i>et al. J. Med. Chem.</i> ; published online March 23, 2009; doi:10.1021/jm801640k Contact: Timor Baasov, Technion-Israel Institute of Technology, Haifa, Israel e-mail: chtimor@tx.technion.ac.il
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