This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Amyloidosis	Transthyretin (TTR)	<i>In vitro</i> and mouse studies identified two TTR-stabilizing molecules that could help prevent TTR-mediated amyloidosis. Aggregates and amyloid deposits of wild-type or mutant forms of TTR can lead to various forms of amyloidosis. In a TTR aggregation assay, the stabilizers 4ajm15 and mds84 prevented aggregation of both wild-type and a mutant form of TTR compared with vehicle. Next steps could include further optimization of the compounds to improve their pharmacokinetic and toxicology profiles. Tafamidis, a small molecule from Pfizer Inc. that stabilizes TTR protein and prevents misfolding, is in Phase II/III testing for amyloidosis. ALN-TTR01, a stable nucleic acid lipid particle (SNALP)-formulated RNAi from Alnylam Pharmaceuticals Inc. and Tekmira Pharmaceuticals Corp. that targets the <i>TTR</i> gene, is in Phase I testing for amyloidosis. SciBX 3(45); doi:10.1038/scibx.2010.1360 Published online Nov. 18, 2010	Patents pending for the two stabilizing compounds; exclusively licensed to an undisclosed company	Kolstoe, S.E. <i>et al.</i> <i>Proc. Natl. Acad. Sci. USA</i>; published online Nov. 11, 2010; doi:10.1073/pnas.1008255107 Contact: Mark B. Pepys, University College London, London, U.K. e-mail: m.pepys@medsch.ucl.ac.uk