This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Cardiomyopathy	Transthyretin (TTR)	<p><i>In vitro</i> and cell culture studies identified small molecule TTR stabilizers that could help treat cardiomyopathy caused by TTR aggregation. In a human cardiomyocyte assay of TTR-driven cardiotoxicity, several different TTR-binding compounds decreased TTR amyloid deposition and increased cell viability compared with vehicle. Next steps include optimizing the lead and comparing the new compounds against TTR-stabilizing compounds in clinical development. In April, the FDA issued a refusal to file letter for an MAA for Pfizer Inc.'s Vyndaqel tafamidis to treat TTR amyloidosis.</p> <p>Isis Pharmaceuticals Inc. and Alnylam Pharmaceuticals Inc. respectively have antisense and RNAi therapeutics targeting TTR in Phase I testing for amyloidosis.</p> <p>SciBX 4(36); doi:10.1038/scibx.2011.1018 Published online Sept. 15, 2011</p>	Patents filed for composition of matter and screening methods; available for licensing	<p>Alhamadesheh, M.M. <i>et al. Sci. Transl. Med.</i>; published online Aug. 24, 2011; doi:10.1126/scitranslmed.3002473</p> <p>Contact: Isabella A. Graef, Stanford University Medical School, Stanford, Calif. e-mail: igraef@stanford.edu</p>