This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
α_1 -Antitrypsin deficiency	α_1 -Antitrypsin (SERPINA1; AAT; A ₁ AT)	<p><i>In vitro</i> and mouse studies suggest the small molecule carbamazepine could be useful for treating AAT deficiency, a disorder characterized by the accumulation of pathogenic protein aggregates in liver cells and subsequent liver fibrosis and cancer. In human cell lines expressing mutant AAT, the autophagy inducer carbamazepine decreased mutant protein levels compared with no treatment. In a mouse model of AAT deficiency, carbamazepine decreased hepatic loads of the mutant protein and reduced fibrosis compared with vehicle control. Next steps include clinical trials of the compound to treat AAT deficiency.</p> <p>Novartis AG markets Tegretol carbamazepine to treat epilepsy.</p> <p>Shire plc and Impax Laboratories Inc. market Carbatrol carbamazepine, a sustained-release formulation, to treat epilepsy and seizures.</p> <p>SciBX 3(24); doi:10.1038/scibx.2010.735 Published online June 17, 2010</p>	Provisional patent application filed in the U.S. for use of an anticonvulsant to treat AAT deficiency and other diseases caused by aggregation-prone proteins; available for licensing	<p>Hidvegi, T. <i>et al. Science</i>; published online June 3, 2010; doi:10.1126/science.1190354</p> <p>Contact: David H. Perlmutter, Children's Hospital of Pittsburgh of The University of Pittsburgh School of Medicine, Pittsburgh, Pa. e-mail: david.perlmutter@chp.edu</p> <p>Contact: Marc Lukasiak, same affiliation as above e-mail: marc.lukasiak@chp.edu</p> <p>Contact: Anita Srikameswaran, same affiliation as above e-mail: srikamav@upmc.edu</p>