This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mitochondrial disease	Mammalian target of rapamycin (mTOR; FRAP; RAFT1)	Mouse studies suggest inhibiting mTOR with rapamycin could help treat Leigh's disease. In a mouse model of the mitochondrial disorder Leigh's disease, daily i.p. administration of 8 mg/kg rapamycin extended survival and delayed neurological symptoms, and also decreased both disease progression and neuroinflammation compared with vehicle. In the mouse model, rapamycin also decreased levels of glycolytic intermediates typically elevated in Leigh's syndrome compared with vehicle and induced a shift toward amino acid catabolism. Next steps include determining safety and efficacy of rapamycin or rapamycin analogs in young patients with Leigh's disease. *SciBX* 7(1); doi:10.1038/scibx.2014.19 Published online Jan. 9, 2014	Unpatented; licensing status not applicable	Johnson, S.C. *et al. Science*; published online Nov. 14, 2013; doi:10.1126/science.1244360 **Contact:** Matt Kaeberlein, University of Washington, Seattle, Wash. e-mail: kaeber@uw.edu