This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Hyperoxaluria	Alanine-glyoxylate aminotransferase (AGXT)	*In vitro* studies suggest dequalinium chloride (DECA) could help treat primary hyperoxaluria (PH1), a disease caused by *AGXT* mutations. AGXT functions in the peroxisome to detoxify glyoxalate and prevent oxalate accumulation, but at least one *AGXT* mutation diverts the enzyme to the mitochondria and prevents its normal function. In a mammalian cell line expressing the *AGXT* mutation, the generic antibacterial agent DECA decreased enzyme entry in the mitochondria and increased enzyme trafficking to the peroxisome compared with vehicle. In the mammalian cell line challenged with glycolate, DECA decreased oxalate levels comparably to pyridoxine, a compound effective in some patients with PH1. Next steps include testing DECA in animal models of PH1.	Patent application filed; available for licensing	Miyata, N. *et al. Proc. Natl. Acad. Sci. USA*; published online Sept. 18, 2014; doi:10.1073/pnas.1408401111 **Contact:** Carla M. Koehler, University of California, Los Angeles, Calif. e-mail: koehler@chem.ucla.edu

SciBX 7(40); doi:10.1038/scibx.2014.1179
Published online Oct. 16, 2014