

## THE DISTILLERY

## This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Metabolic disease				
Mitochondrial disease	Creatine (Cr)	Studies in cell culture and in human plasma suggest that creatine could help diagnose inherited mitochondrial respiratory-chain diseases. In cultures of human muscle cells, metabolic profiling of cells treated with respiratory-chain inhibitors identified 32 metabolites significantly affected by the inhibitors compared with profiling of cells treated with vehicle ( $p$ <0.05). In plasma samples from two separate cohorts of patients with respiratory chain diseases, higher levels of creatine correlated more strongly with disease than did other levels of metabolites, including lactate, an established disease marker ( $p$ <0.05). Planned studies include metabolic profiling of mitochondrial dysfunction in larger human cohorts. Edison Pharmaceuticals Inc. and Penwest Pharmaceuticals Co's $\alpha$ -tocopherol quinone (A0001), a coenzyme Q10 analog that increases electron flux and ATP synthesis, is in Phase I/IIa testing to treat inherited mitochondrial respiratory chain diseases.	Patented by Massachusetts General Hospital; available for licensing	Shaham, O. <i>et al. Proc. Natl. Acad. Sc.</i> <i>USA</i> ; published online Dec. 28, 2009; doi:10.1073/pnas.0906039107 <b>Contact:</b> Vamsi K. Mootha, Harvard Medical School, Boston, Mass. e-mail: vamsi@hms.harvard.edu

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