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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Mitochondrial disease	Pyruvate dehydrogenase kinase (PDK)	*In vitro* and *in vivo* studies suggest phenylbutyrate could help treat mitochondrial pyruvate dehydrogenase complex (PDHC) deficiency, the most common genetic form of lactic acidosis. In enzymatic assays, phenylbutyrate bound PDK to prevent inactivation of PDHC. In 9 of 15 patient-derived, PDHC-deficient fibroblast cell lines, phenylbutyrate increased PDHC activity compared with no treatment. In zebrafish and mouse models for lactic acidosis, phenylbutyrate decreased lactate levels compared with no treatment. Next steps include designing a clinical trial for patients with PDHC deficiency. Valeant Pharmaceuticals International Inc. and Hyperion Therapeutics Inc. market Ravicti glycerol phenylbutyrate to treat urea cycle disorder. The compound is in Phase II testing to treat liver disease.	Unpatented; unlicensed	Ferriero, R. *et al. Sci. Transl. Med.*; published online March 6, 2013; doi:10.1126/scitranslmed.3004986 **Contact:** Nicola Brunetti-Pierri, Telethon Institute of Genetics and Medicine, Naples, Italy e-mail: brunetti@tigem.it

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