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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Porphyria	Proteasome; uroporphyrinogen III synthase (UROS)	Mouse and cell culture studies suggest proteasome inhibitors could be useful for treating congenital erythropoietic porphyria, which is caused by destabilizing mutations in *UROS*. In human erythrocyte cell lines carrying mutations in *UROS* that destabilize the protein, the proteasome inhibitor Velcade bortezomib partially protected the synthase from premature degradation. In mice carrying analogous *Uros* mutations, Velcade decreased disease severity compared with saline. Next steps could include evaluating other proteasome inhibitors in the preclinical models and testing various dosing protocols. Takeda Pharmaceutical Co. Ltd. and Johnson & Johnson market Velcade to treat multiple myeloma (MM) and mantle cell lymphoma (MCL). Amgen Inc. and Ono Pharmaceutical Co. Ltd. market the selective proteasome inhibitor Kyprolis carfilzomib to treat MM. At least 10 companies have proteasome inhibitors in Phase III testing or earlier to treat various cancers.	Patent and licensing status unavailable	Blouin, J.-M. *et al. Proc. Natl. Acad. Sci. USA*; published online Oct. 21, 2013; doi:10.1073/pnas.1314177110 **Contact:** Emmanuel Richard, University of Bordeaux Segalen, Bordeaux, France e-mail: emmanuel.richard@u-bordeaux2.fr

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