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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Parkinson's disease (PD)	Ubiquitin carboxyl-terminal esterase L1 (UCHL1); α-synuclein (SNCA)	Studies in cell culture suggest that inhibiting a membrane-bound form of UCHL1 could be useful for treating PD. In cultured human neuroblastoma cells, levels of a farnesylated, membrane-bound UCHL1 directly correlated with neurotoxicity of SNCA, a protein associated with PD. In cell culture models of SNCA neurotoxicity, inhibition of UCHL1 with the farnesyl transferase inhibitor FT1-277 significantly lowered the toxicity and accumulation of SNCA compared with what was seen in controls ($p<0.05$). Next steps could include determining the therapeutic potential of inhibiting UCHL1 farnesylation in animal models of PD. In 2005, Johnson & Johnson received a not approvable letter for Zarnestra tipifarnib (R115777), a farnesyl transferase inhibitor used to treat elderly patients with newly diagnosed acute myeloid leukemia (AML).	Patent and licensing status undisclosed	Liu, Z. *et al. Proc. Natl. Acad. Sci. USA*; published online Feb. 23, 2009; doi:10.1073/pnas.0806474106 **Contact:** Peter T. Lansbury, Jr., Link Medicine Corporation, Cambridge, Mass. e-mail: peter@linkmedicine.com

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