



This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Alzheimer's disease (AD); neurology	3-Phosphoinositide-dependent protein kinase-1 (PDPK1)	Mouse and cell culture studies suggest inhibiting PDPK1 activity could help treat AD and prionassociated diseases. In prion-infected, cultured mouse neurons and neurons from mice with amyloid plaques, a PDPK1 inhibitor led to higher levels of neuroprotective amyloid precursor protein (APP) and cellular prion protein (PRNP; PrP; CD230) cleavage products than no treatment. In mouse models, the PDPK1 inhibitor decreased both AD-specific behavioral impairments and prion disease–specific motor impairments compared with no treatment. Next steps include testing other PDPK1 inhibitors and analyzing how PDPK1 is activated by pathogenic prions and amyloid peptides. Arno Therapeutics Inc.'s AR-12, a small molecule inhibitor of PDPK1, is in Phase I testing to treat lymphoma and solid tumors. Phusis Therapeutics Inc.'s PHT-427, a small molecule inhibitor of PDPK1, is in preclinical development to treat cancer.	Patent and licensing status undisclosed	Pietri, M. et al. Nat. Med.; published online Aug. 18, 2013; doi:10.1038/nm.3302 Contact: Benoit Schneider, University Paris Descartes, Paris, France e-mail: benoit.schneider@parisdescartes.fr Contact: Jean-Marie Launay, Lariboisière Hospital, Paris, France e-mail: jean-marie.launay@lrb.aphp.fr
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