

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	AMP-activated protein kinase (AMPK)	Cell culture studies suggest inhibiting AMPK signaling could help treat SOD1-driven ALS. About 15% of familial ALS cases involve mutations in <i>superoxide dismutase 1</i> (SOD1). In cultured rat motor neurons, an AMPK inhibitor decreased mutant Sod1–induced cell death compared with vehicle. An AMPK activator increased mutant Sod1–induced cell death compared with vehicle. Next steps could include screening for a lead AMPK inhibitor and evaluating the compound in mammalian models of ALS.	Patent and licensing status unavailable	Lim, M.A. <i>et al. J. Neurosci.</i> ; published online Jan. 18, 2012; doi:10.1523/ JNEUROSCI.6554-10.2012 Contact: Robert G. Kalb, The Children's Hospital of Philadelphia, Philadelphia, Pa. e-mail: kalb@email.chop.edu

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