

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Amyotrophic lateral sclerosis (ALS)	Protease-activated receptor 1 (PAR1)	Studies in mice suggest that PAR1 agonists could help treat familial ALS. In a mouse model of superoxide dismutase 1 (SOD1)-driven ALS, treatment with a modified form of the PAR1 ligand activated protein C (APC) reduced disease severity and increased survival compared with mock treatment. Next steps include engineering APC variants that minimize off-target effects on blood coagulation. About 20% of familial ALS cases involve mutations in SOD1. Xigris drotrecogin alfa, a recombinant human APC from Eli Lilly and Co., is marketed to treat sepsis.	Covered by issued and pending patents from the University of Rochester, The Scripps Research Institute and ZZ Biotech LLC	Zhong, Z. et al. J. Clin. Invest.; published online Oct. 19, 2009; doi:10.1172/JCI38476 Contact: Berislav V. Zlokovic, University of Rochester Medical Center, Rochester, N.Y. e-mail: berislav_zlokovic@urmc.rochester.edu

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