

THE DISTILLERY

This week in therapeutics

| Indication | Target/marker/pathway | Summary | Licensing status | Publication and contact information |
|-------------------------------------------|----------------------------------|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|--------------------------------------------|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Neurology | | | | |
| Amyotrophic lateral sclerosis (ALS) | Superoxide dismutase 1 (SOD1) | Mouse studies suggest increasing the copper content of the SOD1 metalloprotein could be useful for treating ALS. In a mutant SOD1 mouse model of ALS, daily oral treatment with diacetyl- <i>bis</i> (4-methylthiosemicarbazonato) copper(II) (Cu(II)(atsm)) improved locomotor function and increased survival compared with vehicle. In spinal cords from these mice, Cu(II) (atsm) increased the copper content of mutant SOD1 compared with vehicle, suggesting that copper deficiency could underlie the protein's neurotoxicity. Next steps could include developing a screen for compounds that increase SOD1 copper content. At least four companies have compounds that target SOD1 in preclinical development to treat ALS. Procypra Therapeutics LLC has derivatives of Cu(II)(atsm) in discovery to treat Parkinson's disease (PD). | Patent and licensing status unavailable | Roberts, B.R. <i>et al. J. Neurosci.</i> ; published online June 4, 2014; doi:10.1523/JNEUROSCI.4196-13.2014 Contact: Peter J. Crouch, The University of Melbourne, Melbourne, Victoria, Australia e-mail: pjcrouch@unimelb.edu.au |
| | | <i>SciBX</i> 7(27); doi:10.1038/scibx.2014.799 Published online July 17, 2014 | | |