This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Neurology				
Leukodystrophy	Proteolipid protein 1 (PLP1)	Mouse and patient studies suggest human neural stem cells could help treat hypomyelination diseases. In a mouse model of hypomyelination, human neural stem cells transplanted into three different sites of neonatal or juvenile mouse brains differentiated into oligodendrocytes and myelinated axons. Brain slices from mice receiving transplants showed longer duration for action potentials than mice receiving vehicle, indicating functional myelination. In four juvenile patients with Pelizaeus-Merzbacher disease, a leukodystrophy caused by mutations in *PLP1*, transplantation of human neural stem cells into the frontal lobe white matter plus nine months of immunosuppression led to modest gains in neurological function and motor performance. Magnetic resonance spectroscopy indicated increased axon myelination at one year postsurgery. Next steps could include Phase II clinical testing. *SciBX* 5(42); doi:10.1038/scibx.2012.1120 Published online Oct. 25, 2012	Findings in both papers patented by StemCells Inc.; available for licensing	Uchida, N. *et al. Sci. Transl. Med.*; published online Oct. 10, 2012; doi:10.1126/scitranslmed.3004371 **Contact:** Stephen A. Back, Oregon Health & Science University, Portland, Ore. e-mail: backs@ohsu.edu **Contact:** Nobuko Uchida, StemCells Inc., Newark, Calif. e-mail: nobuko.uchida@stemcellsinc.com Gupta, N. *et al. Sci. Transl. Med.*; published online Oct. 10, 2012; doi:10.1126/scitranslmed.3004373 **Contact:** David H. Rowitch, University of California, San Francisco, Calif. e-mail: rowitchd@peds.ucsf.edu