This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Spinal muscular atrophy (SMA)	Plastin 3 (PL3)	A study in animal models suggests that upregulating PL3 could be useful for treating SMA. Homozygous deletion of the gene encoding survival motor neuron gene 1 (SMN1) causes SMA. In a mouse SMA model with reduced SMN1 expression, PL3 overexpression significantly restored axonal length compared with that seen in mice who received mock expression vector or GFP control protein ($p<0.0004$). In a zebrafish SMA model, antisense SMN knockdown in combination with PL3 overexpression significantly reduced aberrant axonal outgrowth compared with the results of SMN knockdown alone ($p<0.0004$). Next steps include generating transgenic mice that overexpress PL3 and crossbreeding them with SMA mice to determine whether the disease phenotype is rescued or improved in a mammalian system.	Not patented; unlicensed	Oprea, G. *et al. Science*; published online April 25, 2008; doi:10.1126/science.1155085 **Contact:** Brunhilde Wirth, University of Cologne, Cologne, Germany e-mail: brunhilde.wirth@uk-koeln.de