This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular atrophy	Androgen receptor; insulin-like growth factor-1 (IGF-1)	Studies in cell culture and in mice suggest that IGF-1 could treat spinal and bulbar muscular atrophy (SBMA). In multiple cell lines expressing a mutant androgen receptor associated with SBMA, exogenous IGF-1 reduced receptor aggregation compared with vehicle control. Aberrant aggregation of mutant receptors underlies disease pathology. In a mouse model of SBMA, animals with skeletal muscle–specific overexpression of Igf-1 had lower androgen receptor aggregation and muscle and spinal cord pathology and greater survival than mice that did not express Igf-1. Ongoing work is seeking to elucidate the signaling pathways by which IGF-1 inhibits SBMA disease progression. Ipsen Group markets Increlex mecasermin, an injectable formulation of recombinant human IGF-1, to treat children with severe primary IGF-1 deficiency (IGFD). *SciBX* 2(32); doi:10.1038/scibx.2009.1246 Published online Aug. 20, 2009	Unpatented; unlicensed	Palazzolo, I. *et al. Neuron*; published online Aug. 12, 2009; doi:10.1016/j.neuron.2009.07.019 **Contact:** Maria Pennuto, Italian Institute of Technology, Genoa, Italy e-mail: maria.pennuto@iit.it