This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy; Duchenne muscular dystrophy	Cyclophilin D	Studies in mice suggest that antagonizing cyclophilin D could treat muscular dystrophy. Cyclophilin D is a mitochondrial matrix protein involved in regulating mitochondrial permeability and cellular necrosis. In murine δ-sarcoglycan knockout models of severe muscular dystrophy, mice with additional knockout of cyclophilin D showed significantly greater muscle mass than mice that expressed cyclophilin D ($p<0.05$). In murine dystrophin knockout models of Duchenne muscular dystrophy, the cyclophilin inhibitor Debio-025 reduced muscle fibrosis whereas treatment with vehicle did not. The researchers said they are in discussions with Debiopharm S.A. regarding a clinical trial of Debio-025 to treat muscular dystrophy. Debiopharm's product is in Phase II testing to treat HCV infection.	Patent and licensing status undisclosed	Millay, D. *et al. Nat. Med.*; published online March 16, 2008; doi:10.1038/nm1736 **Contact:** Jeffery D. Molkentin, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio e-mail: jeff.molkentin@cchmc.org