

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal d	isease			
Muscular atrophy; muscular dystrophy	Myostatin (GDF-8)	The X-ray crystal structure of myostatin complexed with the antagonist follistatin 288 (Fst288) could aid the development of therapies for muscular atrophy and muscular dystrophy. The structure showed how the binding of myostatin to follistatin increases myostatin's affinity for heparin, which leads to greater myostatin degradation. Ongoing research involves using the structural information to rationally design myostatin inhibitors. AMG 745, a myostatin inhibitor from Amgen Inc., is in Phase I testing to treat muscular atrophy. ACE-031, a myostatin inhibitor from Acceleron Pharma Inc., is in Phase I testing to treat muscular dystrophy.	Work unpatented; structure freely available from the Protein Data Bank (PDB)	Cash, J.N. <i>et al. EMBO J.</i> ; published online July 30, 2009; doi:10.1038/emboj.2009.205 Contact: Thomas B. Thompson, National Institutes of Health, Bethesda, Md. e-mail: thompstb@ucmail.uc.edu

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