

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Musculoskeletal disease				
Muscular dystrophy	<i>DAZA binding element transcript (DBE-T)</i>	Cell culture studies suggest inhibiting <i>DBE-T</i> could help treat facioscapulohumeral dystrophy (FSHD), which is a common autosomal-dominant type of muscular dystrophy. Earlier studies have shown that patients with FSHD have reductions in the copy number of the <i>D4Z4</i> repeats, which leads to reduced repression of disease-associated genes. In cultured muscle cells from FSHD patients, a long noncoding RNA called <i>DBE-Transcript</i> was expressed and associated with <i>D4Z4</i> , whereas <i>DBE-T</i> was not expressed in muscle cells from healthy controls. In cells expressing <i>DBE-T</i> , small hairpin RNA against <i>DBE-T</i> decreased FSHD-associated gene expression compared with control shRNA. Next steps include testing knockdown of <i>DBE-T</i> in animal models.	Unpatented; available for collaboration	Cabianca, D.S. <i>et al. Cell</i> ; published online April 26, 2012; doi:10.1016/j.cell.2012.03.035 Contact: Davide Gabellini, San Raffaele Scientific Institute, Milan, Italy e-mail: gabellini.davide@hsr.it
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