## This week in therapeutics

| Indication | Target/marker/ pathway | Summary | Licensing status | Publication and contact information |
|---|---|---|---|---|
| **Musculoskeletal disease** | | | | |
| Duchenne muscular dystrophy (DMD) | Dystrophin (DMD); nitric oxide synthase 1 (neuronal) (NOS1; nNOS) | *Ex vivo* and mouse studies suggest that a new synthetic *dystrophin* gene could treat DMD. In *ex vivo* skeletal muscle fibers, two regions of *DMD*'s rod domain—R16 and R17—were shown to be essential to nNOS's localization to muscle cell membranes. In the dystrophin-deficient *mdx* mouse model of DMD, a synthetic *DMD* microgene encoding R16 and R17 decreased muscle pathology, increased muscle strength and exercise performance, and improved muscular NO diffusion compared with what was seen in untreated controls. Ongoing work is seeking to elucidate the mechanism of nNOS-R16/R17 interactions and identify a synthetic *DMD* gene suitable for use in larger animal models.<br>At least six companies have therapies in preclinical to early-stage clinical testing to treat DMD. | Patented; available for licensing | Lai, Y. *et al. J. Clin. Invest.*; published online Feb. 23, 2009; doi:10.1172/JCI36612<br>**Contact:** Dongsheng Duan, University of Missouri, Columbia, Mo.<br>e-mail: duand@missouri.edu |

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