

This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug platforms			
Genetically corrected induced pluripotent stem (iPS) cell-derived mesoangioblasts for limb-girdle muscular dystrophy	Genetically corrected mesoangioblasts derived from iPS cells could be useful for treating limb-girdle muscular dystrophy, which is caused by mutations in the <i>sarcoglycan-α</i> (SGCA) gene. Fibroblasts and myoblasts were extracted from patients with limb-girdle muscular dystrophy, reprogrammed into iPS cells, differentiated into mesoangioblast-like cells and then genetically corrected <i>ex vivo</i> using a lentiviral vector encoding normal human SGCA. In a mouse model of limb-girdle muscular dystrophy, transplantation of the genetically corrected mesoangioblasts decreased markers of muscular dystrophy and improved motor performance compared with no transplantation. Next steps could include long-term safety and tumorigenicity studies of the cells in large animal models.	Patent pending; available for licensing from San Raffaele Hospital Contact: Paola Pozzi, San Raffaele Hospital, Milan, Italy e-mail: pozzi.paola@hsr.it	Tedesco, F.S. <i>et al. Sci. Transl. Med.</i> ; published online June 27, 2012; doi:10.1126/scitranslmed.3003541 Contact: Francesco Saverio Tedesco, University College London, London, U.K. e-mail: f.s.tedesco@ucl.ac.uk Contact: Giulio Cossu, same affiliation as above e-mail: g.cossu@ucl.ac.uk
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