**How muscle-generating stem cells could treat muscular dystrophy**

By [Tim](http://blogs.terrapinn.com/total-biopharma/author/timothy/) / 8 March 2013 / [0 comments](http://blogs.terrapinn.com/total-biopharma/2013/03/08/muscle-generating-stem-cells-treat-muscular-dystrophy/#respond)



Duchenne Muscular Dystrophy (DMD) may be treatable with stem cell-based therapy, [research has shown](http://www.eurekalert.org/pub_releases/2013-03/uoma-uom030513.php). Utilising a variety of groundbreaking techniques, researchers at the University of Minnesota generated stem cells capable of muscle regeneration in a mouse model for DMD.

DMD is a form of muscular dystrophy where a defective gene fails to encode for the muscle dystrophin protein, and thus patients suffer from a rapid weakening if their musculoskeletal system.

The research, [published in *Nature Communications*](http://www.nature.com/ncomms/journal/v4/n3/full/ncomms2550.html), combined induced pluripotent stem cell technology and a genetic correction tool to create the muscle-generating stem cells.

First of all, researchers took skin cells from mice with mutations in the dystrophin and utrophin genes. These skin cells were then reprogrammed into pluripotent stem cells.

The phenotype of the dystrophic induced pluripotent stem cells was then corrected utilising the second piece of groundbreaking technology – the *Sleeping Beauty*Transposon. *Sleeping Beauty* is a genetic correction tool that can deliver useful genes into the human genome. In this case, *Sleeping Beauty* delivered a gene called “micro-utrophin” into the dystrophic pluripotent stem cells.

Micro-utrophin is similar to dystrophin in that it can support muscle fibre strength throughout the body, but unlike dystrophin that appears foreign to the immune system in DMD, the micro-utrophin appears “invisible”.

In a third groundbreaking move, these corrected dystrophic induced pluripotent stem cells were then coaxed into differentiating into skeletal muscle progenitors by delivering them a short pulse of muscle stem cell protein called Pax3. The Pax3-induced progenitors were then transplanted back into the dystrophic mice from which the pluripotent stem cells originally came from and were thus histocompatible.

The transplanted cells performed well in the dystrophic mice, with the engrafted muscles displaying large numbers of micro-utrophin-positive myofibres and improved contractile strength.

“We were pleased to find the newly formed myofibers expressed the markers of the correction, including utrophin,” [said Rita Perlingeiro](http://www.eurekalert.org/pub_releases/2013-03/uoma-uom030513.php), Ph.D, the principal investigator. “However, a very important question following transplantation is if these corrected cells would self-renew, and produce new muscle stem cells in addition to the new muscle fibers.”

Indeed, the research showed the cells did respond properly to injury, with injured transplanted muscle being able to repair itself.

Although this research was performed in mice, it does pave the way for a similar therapy to be developed for humans with muscular dystrophy that uses genetically corrected autologous induced pluripotent stem cells.

“Utilizing corrected induced pluripotent stem cells to target this specific genetic disease proved effective in restoring function,” [said Antonio Filareto](http://www.eurekalert.org/pub_releases/2013-03/uoma-uom030513.php), Ph.D., a postdoctoral fellow in Perlingeiro’s laboratory and the lead author on the study. “These are very exciting times for research on muscular dystrophy therapies.”

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If you’d like to hear more about innovations and strategy in regenerative medicine, you might be interested in attending the [World Stem Cells & Regenerative Medicine Congress](http://www.terrapinn.com/2013/stemcells/index.stm) 21-23 May 2013, London. Click here to[download the brochure](http://www.terrapinn.com/template/live/documents.aspx?e=5576).

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