

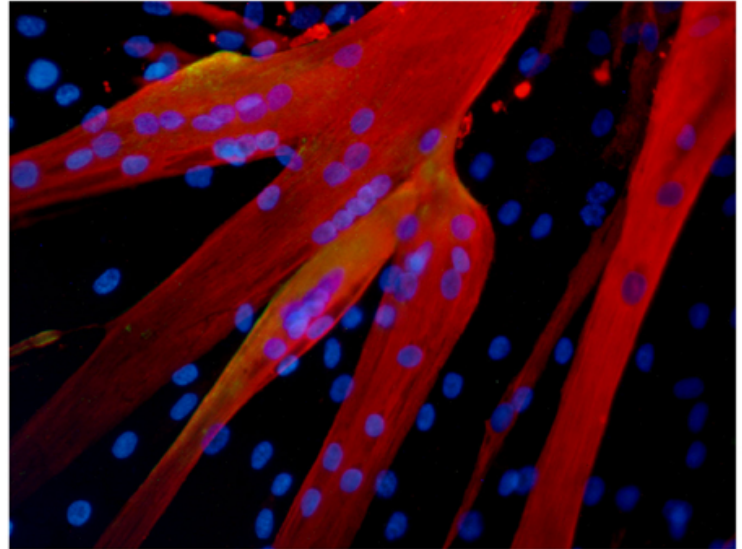
# Muscular dystrophy: how could stem cells help?

## What do we know?

Muscular dystrophies are a group of genetic diseases causing weakness and progressive decline of heart and skeletal muscles.

People with Duchenne muscular dystrophy (DMD) lack a protein called dystrophin, which makes their muscles easily damaged. Muscle damage may lead to inflammation that causes further damage to muscle tissue.

Normally, muscle stem cells, called 'satellite cells', create myoblast cells that repair damaged muscle fibres. However, satellite cells in DMD patients struggle to make enough myoblasts and quickly become depleted.



Muscle fibres formed in the lab by human mesoangioblasts.

Image: courtesy of OptiStem and by Giulio Cossu

## What are researchers investigating?

Researchers are investigating many details about satellite cells and the causes of muscle damage as well as treatments that help reduce muscle damage, such as anti-inflammatory treatments.

Studies are examining ways to preserve, and possibly restore, muscle function by transplanting dystrophin-producing cells into patients. These cells could be healthy donor cells or a patient's own cells that have been genetically modified.

Induced pluripotent stem cells (iPSCs) are being studied as an option for making large numbers of cells with healthy dystrophin genes.

## What are the challenges?

The largest challenge for treatments using donor cell transplants is the potential for transplant rejection by a patient's immune system. Treating patients with their own cells (either genetically modified cells or iPSCs) can largely overcome transplant rejection but have other risks.

All the muscles in a patient are weakened and need treatment. Evenly distributing cells to muscles throughout the body is a big challenge for cell therapy treatments.

Cell therapies also have a relatively low success rate for the imbedding of therapeutic cells into damaged muscles (termed 'low engraftment').