EuroSciCon

April 16-17, 2018
Amsterdam, Netherlands

EuroSciCon Joint Event on

Biotechnology, Stem Cell and Molecular Diagnostics

Biochem Mol biol J 2018 Volume: 4
DOI: 10.21767/2471-8084-C2-012

THE CLINICAL EXPERIENCE WITH STEM CELL THERAPIES IN SKELETAL MUSCLE DISEASES

Daniel Skuk
Research Center of the CHU de Quebec, Canada

Stem cell therapy is a potential treatment for genetic and degenerative disorders of the skeletal muscle, among which the most important is Duchenne muscular dystrophy (DMD). To be used in this approach, the cells must be myogenic, that is, they must have the following properties: (1) fuse with the patient's myofibers to induce the expression of therapeutic proteins in them, (2) form new myofibers, and (3) give rise to new muscle specific stem cells (satellite cells). Considering reports of experiments conducted on mice and dogs, the repertoire of cells exhibiting some of the myogenic capacities seems to have expanded in recent years: CD56+ muscle-derived cells, muscle-derived stem cells, CD133+ cells, mesoangioblasts / pericytes, myoendothelial cells, ALDH+ cells, PW1+/Pax7– interstitial cells, and ß-4-integrin+ cells. The clinical studies of stem cell therapy conducted so far showed that, of the four cell types transplanted in patients with DMD, namely CD56+ muscle-derived cells, bone marrow derived cells, CD133+ cells and mesoangioblasts, the only for which there were observed myogenic properties in the clinics were CD56+ muscle-derived cells. In a clinical trial, we allotransplanted CD56+ muscle-derived cells in 1 cm3 of muscle in 9 patients with DMD immunosuppressed with tacrolimus. Four weeks later, we observed restoration of the therapeutic protein (dystrophin) in 3.5% to 26% myofibers. Evidences of small myofiber neoformation and of potentially graft-derived satellite cells were also observed. A 26-years old DMD patient also received cell allotransplantations under tacrolimus immunosuppression in different muscles, restoring dytrophin in 27.5% of myofibers at 1 month and in 34.5% at 18 months. This patient evidenced that our protocol was feasible in large muscles of humans and that long-term expression of donor-derived dystrophin can be obtained under proper immunosuppression. Further improvements are desirable for efficient clinical applications of this strategy and we are currently working on it.

Daniel.Skuk@crchudequebec.ulaval.ca