Myoblast Encapsulation for Novelty Muscular Dystrophy Gene Therapy

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Muscular dystrophies are heritable, heterogeneous, neuromuscular disorders characterized by deterioration and severe atrophy of skeletal muscle. This disease is caused by mutations in proteins forming the link between the cytoskeleton and the basal lamina. As a result of mutations in the dystrophin gene, Duchenne muscular dystrophy patients suffer from progressive muscle atrophy and diminished muscular regenerative capacity. No efficient therapies are available. The evidence that adult stem cells were capable of participating in the regeneration of more than their resident organ led to the development of potential stem cell treatments for degenerative disorder. In the present review, we describe the different types of myogenic stem cells and their possible use for the progression of cell therapy in Duchenne muscular dystrophy. However the current problem with stem cell therapies is the inability of the stem cells to effectively target and survive in the body long enough to carry out their therapeutic task. This project aims to address this problem by supplying the stem cells with a permeable transportation vehicle that supports cell vitality and protects cells from macrophages and antibodies which are usually responsible for killing of transplanted cells. By encapsulating the stem cells in an alginate bead fitted with aptmeres and nanoparticles, it could improve cell to tissue targeting and provide a way to image, track, and manipulate cell movement using MRI and electromagnets.

Awards Won:
Fourth Award of $500