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Potential therapies for muscular dystrophy

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A successful stem cell therapy for muscular dystrophy requires a readily available source of stem cells that can be expanded in sufficient numbers, can be delivered systemically to multiple muscles including deep muscles and can engraft in muscle fibers capable of replacing dystrophin expression robustly while also repopulating the satellite cell niche to provide long-lasting and continuous regeneration to the muscle. Despite the tremendous capacity of satellite cells to endogenously regenerate the skeletal muscle, intramuscular transplantation of myoblasts has been very difficult due to poor survival, migration and engraftment of donor satellite cells/myoblasts in the muscle. Recent reports by several groups have shown that freshly harvested satellite cells showed better engraftment when transplanted directly in the muscle as compared to cultured myogenic cells or myoblasts. The limitation of this approach is that satellite cells constitute only 2-5% of all the myonuclei in the muscle, and thus sufficient numbers of satellite cells to achieve the desired engraftment cannot be obtained from muscles without prior expansion.

We have shown that following acute injury in limb muscles, satellite cells undergo tremendous in vivo expansion to maintain the satellite cell niche and also give rise to a more advanced myogenic population that can in turn fuse and form new myofibers (Ieronimakis et al., 2010). We will discuss approaches to deliver satellite cells and muscle stem cells systemically. We will also discuss pharmaceutical approaches that aid muscle regeneration and can be combined with stem cell therapy to treat muscular dystrophy.

Biography

Morayma Reyes has received her MD/PhD degree from University of Minnesota in 2003. She has published more than 20 papers in reputed journals and is serving as an editorial board member and reviewer of reputed journals. She has been nominated and awarded multiple Junior Faculty Awards.