Satellite Cells and Their Potential for Therapy in Muscular Dystrophies

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The muscular dystrophies are a group of more than 30 genetic diseases with no treatment that can stop or prevent the progression of disease in any form of dystrophy. The current treatment of muscular dystrophies consists mostly of palliative and supportive measures. Of the more than 30 forms of muscular dystrophy, Duchenne muscular dystrophy (DMD) is the most common. Becker muscular dystrophy (BMD) is similar to DMD but is milder due to its late onset and slower progression. The prevalence of DMD and BMD combined (D/BMD) has been reported as 1.3-1.8 cases per 10,000 males in the age range of 5 to 24 years in four states reporting to the Muscular Dystrophy Surveillance Tracking and Research Network (MD STARnet) [1]. These numbers are limited to data collected from only four states (Arizona, Colorado, Iowa and western NY) and only for those patients that were captured by the health system. Thus, the prevalence of D/BMD is likely to be higher than reported. The prevalence and the devastating effects that these diseases have on patients and their families underscore the need to find therapeutic alternatives that restore muscle integrity or stop the progression of disease.

Forms of therapy aimed at restoring muscle integrity currently under investigation are based in genetic, pharmacological, and cellular approaches. In general, the aim of gene therapy is to deliver a copy of the defective gene carried by viral vectors [2,3] or to restore the reading frame (exon skipping) with antisense oligonucleotides [4-8]. Pharmacological agents have been proposed to read through a premature stop codon in the dystrophin gene (such as genaminic or ataluren) or to increase expression of utrophin, an orthologue of dystrophin with high sequence similarity [9,10]. Steroid treatment has proven to be beneficial for Duchenne muscular dystrophy (DMD) patients for quite some time and for certain forms of limb-girdle muscular dystrophy (LGMD), although the mechanism of action is uncertain and the secondary effects are multiple [11-15]. In addition to the anti-inflammatory and immunosuppressant effects, glucocorticoid treatment also increases the expression of utrophin in DMD by enhancing the activity of an internal ribosomal entry site in the 5' UTR of utrophin mRNA [16]. Other effects of glucocorticoids in DMD treatment are currently unknown. The principle of cell therapy is to provide cells with a normal gene and with the capacity to divide and fuse with host fibers. These donor cells must also be able to maintain a sustainable population in the host muscle. The source of cells that can be used for this purpose is very diverse and includes satellite cells, such as bone marrow mesenchymal stem cells, to determine the presence of α2δ1 and the commitment of those cells to the muscle lineage.

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References


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