Regenerative Medicine

Review

筋ジストロフィーの再生医療

Regenerative Medicine for Muscular Dystrophies

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Summary

Duchenne muscular dystrophy (DMD) is an X-linked genetic disease, affecting one in 3,500 newborn boys. DMD is caused by mutations in the gene that encodes the 427-kDa cytoskeletal protein dystrophin. Dystrophin protects the muscle cell membrane from damage during muscle contraction. DMD muscle fibers deficient in dystrophin degenerate and die due to repeated mechanical stress. In the early stage of DMD, skeletal muscle regenerates to maintain muscle mass and force, but gradually loses the regeneration activity. To recover dystrophin expression at the sarcolemma and restore regenerative activity, normal myoblasts were injected into the muscles of DMD boys in the early 1990s. Although no serious side effects of cell transplantation were reported, the results were disappointing. Recently, however, the discovery of iPS cells again stimulated the researchers to develop cell-based therapy for DMD. In this review, comparing the cell therapy with antisense-oligonucleotide-mediated exon-skipping therapy, we summarize the recent progress in the research of iPS cell-mediated cell therapy for DMD, and discuss the problems to be solved for successful cell therapy for DMD.

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