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Allogenic bone marrow-derived mesenchymal stem therapy in Duchenne muscular dystrophy

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Background: Duchenne muscular dystrophy (DMD) is the commonest hereditary muscular dystrophy. It is characterized by progressive muscle leading loss of walking ability and complete wheelchair dependence. Further, disability leads to respiratory failure, which is the common cause of death.

Aim: The aim of the present work is to evaluate the safety and therapeutic efficacy of allogenic bone marrow derived mesenhymal stem cell therapy in Duchenne muscular dystrophy

Subjects & Methods: This study was conducted on 40 myopathic patients, ages ranging from 6-18 years. DMD was documented by family history, history of disease progression, laboratory investigations, muscle biopsy and genetic study. Patients with fixed deformities were excluded. The study group was randomly divided into patient and control groups. Both groups were given traditional treatment (physiotherapy & medical treatment drugs); while the patient group received additionally stem cell treatment. Stem cells were administered in 6 doses 4 weeks apart in a dose of 3×106 cells/kg suspended in 50 ml PBS. Cell suspension was injected locally in 0.5 ml doses intramuscularly. A single systemic injection in a dose of 3 X10 6 cells/kg was given with the sixth intramuscular dose. Follow-up was done using North Star Ambulatory Assessment CHAQ (Child Health Assessment Questionnaire), manual muscle strength testing using Medical Research Council strength scores and functional outcome measures. Scoring was done before and after every month for 12 months. Any complications or adverse effect were recorded.

Results & Conclusions: During the one year follow-up, no serious complications were recorded. Self-limited pain and mild fever were reported for 48 hours after injection. Significant improvement in assessment scores and quality of life questionnaire was seen in the treatment group. This was translated into substantial improvement in ambulation.

Biography

Wael Abo Elkheir has completed his PhD from Cairo University, Egypt. He is the Co-Founder and Board Member of the Egyptian Society for Progenitor Stem Cell Research, a society initiated with the mission of enhancing scientific research and cooperation in the field of stem cell research and regenerative medicine. He is the Director of a number of registered clinical trials in the field of stem cell therapy, especially for neuro-regeneration and musculoskeletal disorders. He has published more than 20 papers in reputed journals.

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