Format: Abstract

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Human Umbilical Cord Mesenchymal Stem Cells in the Treatment of Duchenne Muscular Dystrophy: Safety and Feasibility Study in India.

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Abstract

OBJECTIVE: Duchenne muscular dystrophy (DMD) is a musculo-degenerative disease characterized by lack of dystrophin production with no definite cure available currently. Discarded umbilical cord is a potential source of mesenchymal stem cells which are non-immunogenic and can be used for transplantation in allogenic set ups. Given the regenerative and anti-inflammatory properties of mesenchymal stem cells (MSCs), here we investigated its role in the cellular therapy of DMD patients.

DESIGN: This is a single-blinded study conducted in various hospitals of India situated in Mumbai, Delhi, and Lucknow. Inclusion criteria for enrolling the patients in the study were boys aged between 5 to 18 years, absence of dystrophin in the immunohistochemistry of muscle biopsy and mutation in dystrophin gene in cytogenetic analysis. The exclusion criteria were presence of dystrophin in the muscle biopsy, patients on corticosteroids etc. UC-MSCs (2 millions/kg body weight) were administered through IV and IM injection. Muscle power in muscles of proximal upper limb, distal upper limb, proximal lower limb, distal lower limb, hip flexors, hip extensors, hip abductors, and paraspinal muscles were measured in 11 DMD patients after UC-MSCs transplantation and were followed for up to 3 years (average follow up 1.5 years). 5 DMD patients did not receive any UC-MSCs transplantation and served as the control group.

RESULTS: The treatment group (N = 11 at baseline) had a pretransplantation strength of 3.45 ± 1.0357 and 4.090 ± 0.8312 in muscles of proximal upper limb and distal upper limb respectively. After 1 year (N = 9) these strengths remained stable with an average of 3.78 (1.03) and 4.22 (0.83). In contrast, the control group (N = 5) has a pre-transplantation strength of 3.6 (0.54) and 4 (1) in the proximal and distal upper limb respectively. After 1 year, (N = 5) 3/5 subjects had a slight but not statistically significant decrease in the proximal upper limb, mean 3.0 (1.0) and 5/5 had a lunit decrease in strength, mean 3.0 (1.0). The treatment group had a pre-transplantation strength of 2.0909 ± 0.8312 and 3.1181 ± 0.8738 in muscles of distal and proximal lower limbs respectively. At 1 year (N = 9), 4/9 subjects had a 1 unit increase in strength in the distal lower limb (mean 3.78 (0.97)) and 8/9 subjects had a lunit increase in strength in the proximal lower limb, mean 3.11 (1.05). The control group has a mean of 3.41 (0.54) and 3.0 (1.0) at baseline in the distal and proximal lower limb respectively. By 1 year, 3/5 subjects had a 1 unit decrease (mean 2.8 (0.45)) and 5/5 had a lunit decrease, mean 2.0 (1.0) in distal and proximal lower limb strength. Stability in muscle function was

also achieved in muscles of hip flexors, hip extensors, hip abductors, and paraspinal muscles at one year as compared to untreated group.

CONCLUSION: UC-MSCs administration not only resulted in the stabilization of muscle power but also did not show GVHD or any deleterious effects on the patients and thus may be considered as safe option for treatment of DMD as compared to control untreated group although further larger double-blinded studies are needed.

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