Abstract:

Rationale: Spinal Muscular Atrophy (SMA) is the most common genetic disorder and presents the most common cause of infant mortality. To date, patient management is symptomatic and focuses on improvement of independence and treatment of complications. Stem cell therapy represents a novel therapeutic option for many neurological diseases.

Subjects: This study included 8 patients diagnosed as spinal muscle atrophy type III.

Interventions: Allogenic mesenchymal stem cells (MSCs) were injected in a dose of $1 \times 10^6$/kg intrathecally and a dose of $3 \times 10^6$/kg injected systemically.

Outcomes: The patients showed improvement of GFM score and upgrading of the GFMC grade from Grade V to Grade III in 3 months. Improved quality of life was reflected in improvement of the PEDI scores. Improvement was noticed in respiration. No complications were encountered. Improvement was maintained until date.

Conclusions: Allogenic MSC therapy may present a new safe therapeutic strategy for SMA patients. Controlled clinical trials are recommended to document the efficacy of the procedure.

Keywords: Neuroregeneration, Spinal Muscle Atrophy, Mesenchymal Stem Cells