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Allogeneic mesenchymal stem cell therapy outcomes for three patients with spinal muscular atrophy type 1.

Villanova M, et al. Am J Phys Med Rehabil. 2015.

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Abstract

No effective medical treatment has been documented for spinal muscular atrophy; however, cellular, molecular, and preclinical studies suggest that allogeneic mesenchymal stem cells may play a role. Three children with spinal muscular atrophy type 1 underwent multiple intrathecal and intravenous infusions of mesenchymal stem cells. Their pretreatment, treatment, and posttreatment physical function were quantitated by the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders scale for two patients and documented by video for all three. Infant Test of Neuromuscular Disorders values were 3 before treatment, 10 and 16 during treatment, and 0 and 10 seven and twelve months after treatment was discontinued, respectively. No adverse effects have been noted for at least 44 and 49 mos from onset of treatment, respectively. These data represent the first objective, quantifiable improvements in physical function for any treatment of spinal muscular atrophy. Although the benefits were lost when the therapy was withdrawn, this may be an initial step in establishing mesenchymal stem cells as a safe and effective treatment of spinal muscular atrophy.

PMID 25882135 [PubMed - in process]

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Related citations

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Chung BH, et al. Pediatrics. 2004.

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Martínez-Hernández R, et al. J Pathol. 2013.

[Embryonic stem cell-derived neural stem cells improve spinal muscular atrophy phenotype in mice.](#)

Corti S, et al. Brain. 2010.

[Consensus statement for standard of care in spinal muscular atrophy.](#)

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Wang CH, et al. J Child Neurol. 2007.

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Wadman RI, et al. Cochrane Database Syst Rev. 2012.

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