



TITLE: Treatment for Adult Onset Distal Spinal Muscular Atrophy: Clinical Effectiveness and Guidelines

DATE: 24 August 2010

RESEARCH QUESTIONS:

1. What is the clinical effectiveness of various treatment options for adult onset distal spinal muscular atrophy?
2. What are the guidelines for the treatment of adult onset distal spinal muscular atrophy?

METHODS:

A limited literature search was conducted on key health technology assessment resources, including PubMed, the Cochrane Library (Issue 8, 2010), University of York Centre for Reviews and Dissemination (CRD) databases, ECRI (Health Devices Gold), EuroScan, international health technology agencies, and a focused Internet search. The search was limited to English language articles published between January 1, 2005 and August 12, 2010. No filters were applied to limit the retrieval by study type. Internet links were provided, where available.

The summary of findings was prepared from the abstracts of the relevant information. Please note that data contained in abstracts may not always be an accurate reflection of the data contained within the full article.

RESULTS:

HTIS reports are organized so that the higher quality evidence is presented first. Therefore, health technology assessment reports, systematic reviews, and meta-analyses are presented first. These are followed by randomized controlled trials, non-randomized studies, and evidence-based guidelines.

One relevant non-randomized study was identified pertaining to the treatment of adult onset distal spinal muscular atrophy. No relevant health technology assessment reports, systematic

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reviews, meta-analyses, randomized controlled trials, or evidence-based guidelines were identified. Information that may be of interest, including resources for patients, has been included in the appendix.

OVERALL SUMMARY OF FINDINGS:

Overall, there is limited evidence regarding the treatment of adult onset distal spinal muscular atrophy. The identified non-randomized study found that patients with type III or IV spinal muscle atrophy had increased muscle strength and function when treated with valproate for eight months.¹ This was a case series of seven patients and authors concluded that further research is necessary.

REFERENCES SUMMARIZED:

Health technology assessments

No literature identified.

Systematic reviews and meta-analyses

No literature identified.

Randomized controlled trials

No literature identified.

Non-randomized studies

1. Wehl CC, Connolly AM, Pestronk A. Valproate may improve strength and function in patients with type III/IV spinal muscle atrophy. *Neurology*. 2006 Aug 8;67(3):500-1.
[PubMed:PM16775228](#)

Guidelines and recommendations

No literature identified.

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APPENDIX – FURTHER INFORMATION:

Non-randomized studies

2. Van den Berg-Vos RM, Visser J, Kalmijn S, Fischer K, de Visser M, et al. A long-term prospective study of the natural course of sporadic adult-onset lower motor neuron syndromes. *Arch Neurol*. 2009 Jun;66(6):751-7. [PubMed:PM19506135](#)

Case studies and case series

3. Roos M, Sarkozy A, Chierchia GB, De WP, Schmedding E, Brugada P. Malignant ventricular arrhythmia in a case of adult onset of spinal muscular atrophy (Kugelberg-Welander disease). *J Cardiovasc Electrophysiol*. 2009 Mar;20(3):342-4. [PubMed:PM19175839](#)

Review articles

4. Stavarachi M, Apostol P, Toma M, Cimponeriu D, Gavrilă L. Spinal muscular atrophy disease: a literature review for therapeutic strategies. *J Med Life*. 2010 Jan;3(1):3-9. [PubMed:PM20302191](#)

Additional references

5. Wang CH, Lunn MR. Spinal muscular atrophy: advances in research and consensus on care of patients. *Curr Treat Options Neurol*. 2008 Nov;10(6):420-8. [PubMed:PM18990310](#)
6. Swoboda KJ, Kissel JT, Crawford TO, Bromberg MB, Acsadi G, D'Anjou G, et al. Perspectives on clinical trials in spinal muscular atrophy. *J Child Neurol*. 2007 Aug;22(8):957-66. [PubMed:PM17761650](#)

Information for patients

7. Personal stories - adult onset [Internet]. Stratford upon Avon, Warwickshire: The Jennifer Trust for Spinal Muscular Atrophy; [cited 2010 August 12]. Available from: http://www.jtsma.org.uk/personal_stories_adult_onset.html
8. Leshner B. Spinal muscular atrophy guidebook [Visual presentation on the Internet]. Metro Productions, producers. Richmond (VA): FightSMA; Type IV / Adult Spinal Muscular Atrophy; [cited 2010 Aug 12]. Available from: <http://www.fightsma.org/index.php?spinal-muscular-atrophy-type-4-adult>
9. Siegel IM, Casey P. 101 Hints to help-with-ease for patients with neuromuscular disease. A do-it-yourself owner's guide [monograph of the Internet]. Tucson, AZ : Muscular Dystrophy Association; 2005 [cited 2010 Aug 12]. Available from: <http://www.mda.org/publications/101hints/>