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

Disclaimer: The Health Technology Inquiry Service (HTIS) is an information service for those involved in planning and providing health care in Canada. HTIS responses are based on a limited literature search and are not comprehensive, systematic reviews. The intent is to provide a list of sources of the best evidence on the topic that CADTH could identify using all reasonable efforts within the time allowed. HTIS responses should be considered along with other types of information and health care considerations. The information included in this response is not intended to replace professional medical advice, nor should it be construed as a recommendation for or against the use of a particular health technology. Readers are also cautioned that a lack of good quality evidence does not necessarily mean a lack of effectiveness particularly in the case of new and emerging health technologies, for which little information can be found, but which may in future prove to be effective. While CADTH has taken care in the preparation of the report to ensure that its contents are accurate, complete and up to date, CADTH does not make any guarantee to that effect. CADTH is not liable for any loss or damages resulting from use of the information in the report.

Copyright: This report contains CADTH copyright material and may contain material in which a third party owns copyright. This report may be used for the purposes of research or private study only. It may not be copied, posted on a web site, redistributed by email or stored on an electronic system without the prior written permission of CADTH or applicable copyright owner.

Links: This report may contain links to other information available on the websites of third parties on the Internet. CADTH does not have control over the content of such sites. Use of third party sites is governed by the owners’ own terms and conditions.
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


Guidelines and recommendations
No literature identified.

PREPARED BY:
Health Technology Inquiry Service
Email: htis@cadth.ca
Tel: 1-866-898-8439
APPENDIX – FURTHER INFORMATION:

Non-randomized studies


Case studies and case series


Review articles


Additional references


Information for patients

